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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2014

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____.

Commission file number 001-36183

CELLADON CORPORATION

(Exact name of registrant as specified in its charter)

Delaware	**33-0971591**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
11988 El Camino Real, Suite 650, San Diego, CA	**92130**
(Address of principal executive offices)	**(Zip Code)**

(858) 366-4288
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Name of each exchange on which registered**
Common Stock, par value $0.001 per share	**The NASDAQ Global Market**

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§229.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☒

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒ (Do not check if a smaller reporting company)	Smaller reporting company	☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes ☐ No ☒

The aggregate market value of voting and non-voting common stock held by non-affiliates of the registrant as of June 30, 2014 totaled approximately $264,282,292 based on the closing price of $16.02 as reported by the NASDAQ Global Market.

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of March 13, 2015 was 23,827,918.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement for the registrant's 2015 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days after registrant's fiscal year ended December 31, 2014 are incorporated by reference into Part III of this report.

CELLADON CORPORATION
Form 10-K
For the Fiscal Year Ended December 31, 2014

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PART I

Forward-Looking Statements

This Annual Report on Form 10-K, including the sections entitled "Business," "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations," may contain "forward-looking statements." We may, in some cases, use words such as "anticipate," "believe," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "will," "would" or the negative of those terms, and similar expressions that convey uncertainty of future events or outcomes, to identify these forward-looking statements. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. Forward-looking statements in this Annual Report include, but are not limited to, statements about:

- the success, cost and timing of our product development activities and clinical trials;
- our ability to obtain and maintain regulatory approval for MYDICAR, our companion diagnostic, and any of our future product candidates, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- our ability to obtain funding for our operations, including funding necessary to complete all clinical trials that may potentially be required to file a biologics license application, or BLA, and a Marketing Authorization Application, or MAA, for MYDICAR for the treatment of heart failure for reduced ejection fraction or HFrEF (also referred to as systolic heart failure);
- the commercialization of our product candidates and companion diagnostic, if approved;
- our plans to research, develop and commercialize our product candidates and companion diagnostic;
- our ability to attract collaborators with development, regulatory and commercialization expertise;
- our plans and expectations with respect to preparation for commercial production of MYDICAR, including contractual commitments for commercial manufacturing capacity at one or more commercial suppliers;
- future agreements with Lonza Biologics, Inc. and/or Lonza Houston, Inc., which we collectively refer to as Lonza, and/or Novasep, Inc., or Novasep, and other third parties in connection with the commercialization of MYDICAR, our companion diagnostic and any other approved product;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- the rate and degree of market acceptance of our product candidates and companion diagnostic;
- regulatory developments in the United States and foreign countries;
- the performance of our third-party suppliers and manufacturers;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- our expectations regarding the period during which we qualify as an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or the JOBS Act; and
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates.

These forward-looking statements reflect our management's beliefs and views with respect to future events and are based on estimates and assumptions as of the filing date of this Annual Report and are subject to risks and uncertainties. We discuss many of these risks in greater detail under "Risk Factors." Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise.

ITEM 1. Business

Overview

We are a clinical-stage biotechnology company with industry-leading expertise in the development of cardiovascular gene therapy. We apply our leadership position in the field of gene therapy and calcium dysregulation to develop novel therapies for diseases with high unmet medical needs. Our lead programs target sarco/endoplasmic reticulum Ca^{2+} -ATPase, or SERCA, enzymes, which are a family of enzymes that play an integral part in the regulation of intra-cellular calcium in all human cells. Calcium dysregulation is implicated in a number of important and complex medical conditions and diseases, such as heart failure, which is a clinical syndrome characterized by poor heart function resulting in inadequate blood flow to meet the body's metabolic needs, as well as blood vessel health, diabetes and neurodegenerative diseases. SERCA2a, an enzyme that becomes deficient in patients with heart failure, was scientifically validated as a molecular target for heart failure in the 1990s and became a focus of internal discovery efforts for many large pharmaceutical companies. However, to date, no other company has been successful in targeting SERCA2a using traditional discovery methods.

Our therapeutic portfolio includes both gene therapies and small molecule compounds targeting diseases characterized by SERCA enzyme deficiency. MYDICAR, our most advanced product candidate, uses gene therapy to target SERCA2a. We believe that our gene therapy approach to modulating SERCA2a overcomes the issues encountered by previous efforts and has the potential to provide transformative disease-modifying effects with long-term benefits in patients with heart failure. In addition, we have in-licensed worldwide rights to patents covering an additional gene therapy product opportunity, the membrane-bound form of Stem Cell Factor, or mSCF, for the treatment of cardiac ischemic damage. We have also identified a number of potential first-in-class compounds addressing novel targets in diabetes and neurodegenerative diseases with our small molecule platform of SERCA2b modulators.

We are the first company to enter clinical development with a product candidate, MYDICAR, that selectively targets SERCA2a. We refer to our Phase 1 trial and Phase 2a trial of MYDICAR together as our CUPID 1 trial. In Phase 2a of our CUPID 1 trial, 39 patients with heart failure for reduced ejection fraction, or HFrEF, which is caused by the inability of the heart to pump blood efficiently due to weakening and enlargement of the ventricles, were enrolled in a randomized, double-blind, placebo-controlled trial. MYDICAR was safe and well-tolerated, reduced heart failure-related hospitalizations, improved patients' symptoms, quality of life and serum biomarkers and improved key markers of cardiac function predictive of survival, such as end systolic volume. Based on these results, as well as our previous preclinical studies and clinical trials, we advanced MYDICAR to a 250-patient randomized, double-blind, placebo-controlled international Phase 2b trial in patients with HFrEF, which we refer to as CUPID 2. We completed enrollment of CUPID 2 in February 2014, reached the primary analysis data cutoff in February 2015 and expect to un-blind the data and announce results in late April 2015. If successful, these results, along with other studies, could form the basis for regulatory submissions for approval with the United States Food and Drug Administration, or FDA, and European Medicines Agency, or EMA.

In 2012, we obtained a Special Protocol Assessment, or SPA, whereby the FDA agreed to use time-to-recurrent heart failure-related hospitalizations as the primary endpoint for a MYDICAR Phase 3 pivotal trial. The design of our CUPID 2 trial is similar to the Phase 3 SPA protocol in that it includes the same primary and secondary end points, and the same method of analysis. However, in CUPID 2 we will use a modified intent to treat, or mITT, approach for the analysis of the primary and secondary endpoints. We have adopted an mITT approach for the CUPID 2 trial in order to more accurately reflect intent to treat in the CUPID 2 study, by excluding from the primary analysis clinical events that occurred in subjects prior to being dosed (with MYDICAR or placebo) or from those subjects who were never dosed in the trial. Results from both the mITT and ITT approaches will need to be consistent to demonstrate a treatment effect. In April 2014, the FDA's Center for Biologics Evaluation and Research, or CBER, granted Breakthrough Therapy designation to MYDICAR for reducing hospitalizations for heart failure in patients who test negative for adeno-associated viral vector 1, or AAV1, neutralizing antibodies, are class III or IV heart failure patients under the New York Heart Association,

or NYHA, classification system, and are not in immediate need of a left-ventricular assist device, or LVAD, or heart transplant. The Breakthrough Therapy program is intended to expedite drug development and review of innovative new drugs that are intended to treat serious or life-threatening diseases and for which preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on a clinically significant endpoint. MYDICAR was the first gene therapy product candidate to receive this designation, and the designation indicates that the FDA has determined that the CUPID 1 trial provided preliminary clinical evidence that MYDICAR may demonstrate substantial improvement over available therapies in heart failure patients for which the designation was granted. The FDA has confirmed that the primary efficacy endpoint of time-to-recurrent heart failure-related hospitalizations in the presence of terminal events would be acceptable for a pivotal trial of MYDICAR. This endpoint counts multiple heart failure-related hospitalizations per patient, and adjusts for the occurrence of terminal events and the correlation of multiple events in a single patient. We are using this endpoint in the ongoing CUPID 2 trial and plan to also use it in the next large clinical trial of MYDICAR, subject to regulatory feedback. More recently, the FDA has confirmed its agreement regarding our use of the joint frailty statistical model as a method of analysis for the primary endpoint and that adequate control over type 1 error (false-positive rate) using the joint frailty model under different conditions had been demonstrated based on FDA's review of our extensive simulation studies. In November 2013, the EMA indicated that if MYDICAR demonstrates a substantial and highly significant treatment effect in the advanced heart failure population, a safety database of approximately 205 to 230 MYDICAR-treated subjects may be sufficient for a safety assessment for an MAA for MYDICAR for the treatment of HFrEF. Recently, the EMA has indicated that they are not satisfied that there is adequate control of the type 1 error (false positive rate) with the joint frailty model. In addition, the EMA also indicated that for CUPID 2 to be considered as a single pivotal trial, a more extreme level of statistical significance than 5% would be required. Based on regulatory guidance and our interactions with the FDA and EMA, it is possible that a Phase 3 trial may not be required for marketing approval, however, we are in the planning stages of an international trial (referred to as CUPID-3 or CELL-003) similar to the CUPID 2 design, the size of which will be determined after CUPID 2 results are available. Depending on the outcome of CUPID 2 and the regulatory response to the results, the CUPID 3 trial could serve as an additional trial for regulatory approval or to further characterize MYDICAR's therapeutic profile.

MYDICAR utilizes a recombinant AAV1 serotype, which is a group of adeno-associated viruses, or AAVs, sharing specific antigens, to deliver the gene for the SERCA2a enzyme. We believe AAV1 serotype vectors are particularly well suited for administration to the heart muscle because AAV vectors are safe and are less immunogenic than other viral vectors commonly used in gene therapy. Most people are exposed to wild type AAV (serotype 2) during childhood, without experiencing any symptoms, because AAV causes no disease. In addition, local delivery of AAV1 to the heart requires extremely small quantities to achieve therapeutic effect, which has contributed to the low incidence of side effects in clinical trials to date. We have developed a companion diagnostic to identify the patients who are AAV1 neutralizing antibody, or NAb, negative and therefore eligible for MYDICAR treatment. We believe approximately 40% of patients in the United States are AAV1 NAb negative. In an effort to expand the population of patients who may be eligible for MYDICAR treatment for HFrEF, we are exploring whether plasma exchange can be used to remove AAV1 NAbs from the circulation in advance of MYDICAR administration, and have plans for a possible Phase 1/2 study of MYDICAR in advanced heart failure patients with HFrEF and pre-existing levels of NAb against the AAV1 vector, who would undergo plasma exchange prior to administration of MYDICAR. Plans for this study are pending the outcome of the CUPID 2 trial to further evaluate the benefit-to-risk ratio and further discussions with the FDA.

In 2015, the American Heart Association estimated that there are nearly six million patients currently diagnosed with heart failure in the United States. Despite optimal guideline-directed therapies employing a wide range of pharmacologic, device, and surgical options, many heart failure patients deteriorate over time. The long-term prognosis associated with heart failure is worse than that associated with the majority of cancers, with a mortality rate of approximately 50% at five years following initial diagnosis. There are one million primary heart failure-related hospitalizations and over 280,000 heart failure-related deaths annually in the United States. The

estimated direct cost of heart failure in the United States in 2012 was $60.2 billion, half of which was related to repeated hospitalizations. The one- and six-month readmission rates after heart failure-related hospitalization are close to 25% and 50%, respectively, and there is growing pressure on hospitals to reduce readmissions for heart failure.

We are initially developing MYDICAR to treat HFrEF patients. HFrEF is characterized by a decreased contraction of the heart muscle. We are also investigating MYDICAR for additional indications, including arteriovenous fistula, or AVF, maturation failure, and for the treatment of patients with advanced heart failure who are on an LVAD. In addition, if supported by the CUPID 2 results, we expect to initiate a clinical trial in 2016 for the treatment of diastolic heart failure (heart failure with preserved ejection fraction, or HFpEF), a condition caused by the inability of the heart to relax normally between contractions. MYDICAR has demonstrated activity in preclinical models of this condition.

We hold worldwide rights to MYDICAR in all indications and markets. We plan to commercialize MYDICAR for any approved heart failure indications using a targeted sales force in the United States focused on selected cardiologists, heart failure specialists and other health care providers who treat the majority of heart failure patients. We believe we can maximize the value of our company by retaining certain commercialization rights to our product candidates and, where appropriate, entering into partnerships for specific therapeutic indications and/or geographic territories.

We are also investigating MYDICAR for enhancing the rate of AVF maturation and are completing preclinical work which is necessary to support our investigational new drug, or IND, application for this new indication. Pending completion of this preclinical work and agreement by the FDA, we intend to conduct a Phase 2a trial with MYDICAR in end-stage renal disease patients undergoing surgery for AVF creation. Over 500,000 Americans have end-stage renal disease requiring dialysis and approximately 100,000 fistulae are placed yearly. An AVF, which is a surgically created connection between an artery and a vein, is placed in the arm to provide access for hemodialysis. The access that is created is routinely used for hemodialysis two to five times per week. The AVF has proven to be the most durable, least complicated, and therefore preferred mode of vascular access for hemodialysis. The clinical problem that has resulted from this practice is that following surgery to create the fistula, approximately 50% of fistulae fail to mature to a usable state for hemodialysis. Furthermore, as many as 25% of hospital admissions in the dialysis population have been attributed to vascular access problems, including fistula malfunction and thrombosis. The biology of SERCA2a in both vascular smooth muscle cells, or VSMC, and endothelial cells provides a unique opportunity to potentially positively impact the pathological processes driving fistula failure. The majority of AVF maturation failures have been attributed to rapid proliferation of VSMC, resulting in vascular blockage or occlusion. In preclinical studies, SERCA2a enzyme deficiency has been associated with VSMC proliferation, and increasing SERCA2a activity has been shown to prevent VSMC proliferation and stenosis of injured blood vessels. In addition to stenosis, maturation of AVF requires that the blood vessels dilate to support the increased blood flow during dialysis sessions. MYDICAR increases blood flow in treated vessels, and therefore these effects may aid AVF maturation.

In July 2014, we in-licensed world-wide rights to gene therapy applications for mSCF for treatment of cardiac ischemia from Enterprise Partners Management, LLC, or Enterprise. Our approach with mSCF gene therapy is to recruit and expand resident stem cells, thereby harnessing advances in gene therapy technologies and also expanding the application to those in which cardiac stem cells have shown promise in clinical and preclinical testing. Our ongoing work is aimed at generating clinically acceptable gene therapy vectors in support of potentially conducting a future clinical trial in patients who have suffered cardiac damage, as well as exploration of potential other applications. We believe mSCF has applications in a number of disease areas, particularly cardiovascular conditions and diseases. mSCF induces c-kit+ stem/progenitor cell expansion *in situ*, as well as cardiomyocyte proliferation, which may represent a new therapeutic strategy to reverse adverse remodeling after cardiac injury. In a preclinical setting, mSCF has demonstrated potential improvements in cardiac function and survival following a myocardial infarction. Specifically, these data suggest mSCF gene therapy promoted a regenerative response characterized by an enhancement in cardiac hemodynamic function; an

improvement in survival; a reduction in fibrosis, infarct size and apoptosis; an increase in cardiac c-kit+ progenitor cells recruitment to the injured area; an increase in cardiomyocyte cell-cycle activation; and Wnt/ß-catenin pathway induction.

In July 2014, we entered into a Loan and Security Agreement with Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc. under which we may borrow up to $25.0 million in two tranches. We borrowed the first tranche of $10.0 million on August 1, 2014. We plan to use the proceeds of the Loan and Security Agreement to provide additional funding for the development of MYDICAR, for other development programs in our pipeline and for general corporate purposes. The second tranche of up to $15.0 million can be drawn through June 30, 2015, but only if data from our ongoing CUPID 2 trial supports the continued development of MYDICAR for its Breakthrough Therapy designation to either a Phase 3 clinical trial or for registration for approval.

In October 2014, we entered into a Facility Construction and Commercial Supply Agreement with Lonza, pursuant to which the parties agreed to initiate detailed design planning, or the Detailed Design, for the potential construction of a new commercial viral therapeutics facility in Portsmouth, New Hampshire for the manufacture of MYDICAR drug substance (AAV1/SERCA2a), and in exchange for an upfront reservation fee payable by us to Lonza, Lonza agreed to reserve, for a period of time extendable on payment of specified reservation extension fees, the capital, property and labor resources necessary to enable the initiation of construction of the facility within 75 days of receipt of notice of our decision to exercise the construction trigger and commit to a long-term supply arrangement for MYDICAR. If we exercise the construction trigger, we will be obligated to (i) fund Lonza's construction of the facility through time and event-triggered milestone payments secured by funds deposited by us into an escrow account upon exercise by us of the construction trigger, (ii) upon completion of the facility, fund Lonza's costs for overhead, including personnel reserved for manufacture of MYDICAR at the facility, and (iii) through such overhead funding arrangement, order from Lonza a certain percentage of our and our partners' annual global commercial supply of MYDICAR during the term of the agreement, subject to certain limits and adjustments.

In March 2015, we entered into a Development, Manufacturing and Supply Agreement with Novasep, which superseded our Letter Agreement with Novasep dated December 19, 2014. Under the terms of the agreement, the parties agreed to continue the work initiated under the Letter Agreement, including the work necessary to prepare for the potential manufacture of MYDICAR drug substance (AAV1/SERCA2a) at the facilities of Novasep's affiliate in Europe. Pursuant to the agreement (and as previously agreed in the Letter Agreement), in exchange for payments from us to Novasep, Novasep agreed to (i) conduct the engineering design work for facility modifications that would be necessary for the manufacture of MYDICAR drug substance, (ii) undertake initial process and analytical transfer and initial scale-up work in support of such potential future commercial manufacturing of MYDICAR drug substance, and (iii) allocate the resources and capacity necessary for the foregoing activities. The parties have also agreed to proceed with the additional process transfer, engineering/construction, scale-up and development activities necessary for Novasep's future production of MYDICAR drug substance in accordance with current Good Manufacturing Practices (GMP), and agreed to terms of a commercial supply arrangement with a term through at least December 31, 2018. We have the right to terminate the agreement, exercisable for a specified period of time following the un-blinding of CUPID 2 data, if we conclude in good faith that the CUPID 2 data is such that we do not require production of MYDICAR drug substance at the Novasep Facility. Unless we exercise the post CUPID 2 data termination right described above, we will be obligated to (i) fund Novasep's modifications to the Novasep Facility through time-and event-triggered milestone payments, (ii) make additional payments for the development services to be performed by Novasep, and (iii) commit to purchase a specified number of batches of MYDICAR drug substance (or make minimum payments with respect to any such batches that are not purchased) during the term of the agreement.

Strategy

We are committed to applying our first-mover scientific leadership position in the field of cardiovascular gene therapy and SERCA2 enzymes to transform the lives of patients with debilitating, life-threatening diseases or conditions. Each of our ongoing and planned development projects addresses diseases or conditions with high unmet medical need. The core elements of our strategy include:

- ***Successfully develop MYDICAR as a novel, first-in-class therapy for patients with heart failure with reduced ejection function (HFrEF).*** Based on positive results from our CUPID 1 trial for MYDICAR, we are conducting our CUPID 2 trial to evaluate the safety and efficacy of MYDICAR to reduce heart failure-related hospitalizations in patients with HFrEF. We completed enrollment of this trial in February 2014, reached the primary analysis data cutoff in February 2015 and expect to un-blind the data and announce results in late April 2015. In the United States alone, several hundreds of thousands of patients with HFrEF currently have a poor prognosis and limited treatment options. We believe MYDICAR, if approved, would become a valuable treatment option for these patients.
- ***Advance MYDICAR through an expedited development and review process as a designated Breakthrough Therapy product candidate.*** In 2012, we obtained an SPA in the context of a Phase 3 clinical trial protocol whereby the FDA agreed to the use of time-to-multiple heart failure-related hospitalizations as the primary endpoint for a potential pivotal trial of MYDICAR. Our ongoing CUPID 2 trial uses a similar clinical protocol as was agreed to in the SPA. Following completion of our ongoing CUPID 2 trial, we anticipate that we will have meetings with the FDA and EMA to discuss what remaining clinical trials will be required for approval of MYDICAR. In April 2014, the FDA granted Breakthrough Therapy designation to MYDICAR for reducing hospitalizations for heart failure in patients who test negative for AAV1 NAbs, are NYHA class III or IV heart failure patients and are not in immediate need of an LVAD or heart transplant.
- ***Maximize the value of our MYDICAR franchise by expanding into additional indications.*** The broad therapeutic potential of MYDICAR in multiple indications presents opportunities to maximize the value of our MYDICAR franchise. Beyond our lead proposed indication of HFrEF, we are also developing MYDICAR for additional indications including as treatment of AVF maturation failure and for the treatment of patients with advanced heart failure who are on an LVAD. Also, pending analysis of the CUPID 2 results, we plan to initiate a development program in HFpEF. This condition is also characterized by a SERCA2a deficiency. We may selectively form collaborative alliances to expand and accelerate our development capabilities and product offerings for indications that are poorly managed by existing treatment options.
- ***Commercialize MYDICAR using a highly-targeted cardiology-focused sales force in the United States.*** Heart failure patients are largely treated at leading hospitals and medical centers of excellence by a select group of high-prescribing cardiologists, heart failure specialists and other health care providers. We plan to commercialize MYDICAR for all potential heart failure indications using a targeted sales force focused on these treating physicians. We believe cardiologists, heart failure specialists and interventional cardiologists are typically early adopters of innovative products, devices and technologies, in part because the rate of innovation in this sector has been sustained, and in part because of the large unmet need that their patients exhibit. We believe that MYDICAR would be adopted first by certain cardiologists and heart failure specialists at high-volume, key-opinion-leading hospitals and medical centers, and progressively by a broader segment of the market.
- ***Seek a MYDICAR collaboration partner for territories outside the United States.*** In order to expand our commercial reach and leverage market specific expertise, we will likely seek a collaboration partner for MYDICAR marketing rights outside the United States.
- ***Advance our additional preclinical assets, including our stem cell factor gene therapy and our small molecule platform targeting SERCA2 enzymes.*** We intend to leverage our leading position and proprietary scientific expertise in gene therapy and SERCA2 high throughput screening assays to identify SERCA2 small molecule product candidates and advance mSCF gene therapy towards clinical

testing. We have established early preclinical proof-of-concept results in the fields of heart failure, diabetes and neurodegenerative diseases with these programs. We plan to continue to advance these programs in certain diseases by ourselves or through a partnering strategy.

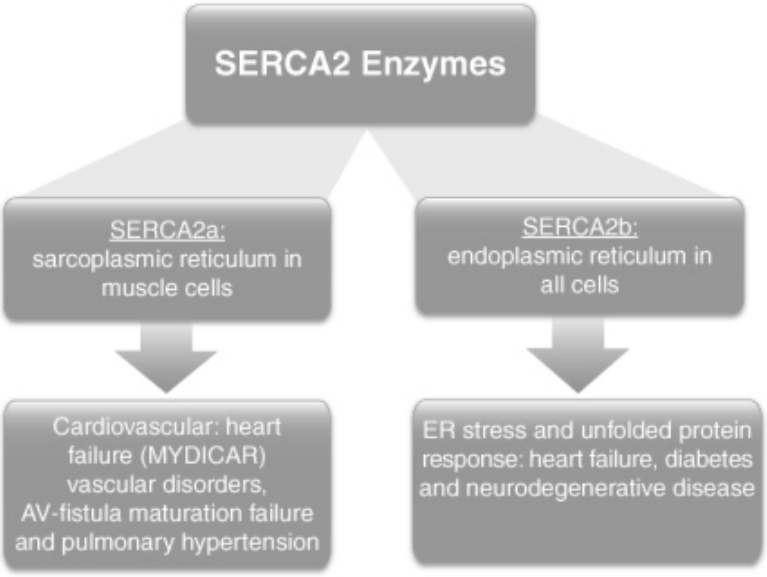
- ***Deploy capital strategically to develop our portfolio of product candidates and create stockholder value.*** We intend to deploy most of our capital resources to further support the manufacture and clinical development of our lead product candidate, MYDICAR. We strive to leverage new clinical design principles and regulatory approval paths to advance our product candidates towards key value inflection points in a capital efficient manner. We believe we can maximize the value of our company by retaining substantial commercial rights to our product candidates and, where appropriate, entering into partnerships for certain indications and/or geographic territories. We believe this combination of independent development and targeted commercialization, together with selective partnering activities, will allow us to capture substantial value of our product candidates while reducing our need for human and capital resources.

Our Platform

We are applying our leading expertise in the field of cardiovascular gene therapies and SERCA2 biology towards the development of therapeutics for significant unmet medical needs. For our SERCA2 program, we are targeting a specific class of proteins, or enzymes, that control calcium levels inside all cells. We believe that SERCA enzymes function as “master switches” that are critical to keeping cells of the body healthy through regulation of calcium levels. SERCA2 enzyme levels are deficient in many disease states, such as heart failure, AVF maturation failure, pulmonary arterial hypertension, or PAH, which is characterized by a SERCA2a deficiency in VSMC, diabetes and neurodegenerative diseases. We believe that the involvement of SERCA2 deficiencies in multiple diseases and conditions creates “franchise” opportunities for our first-in-class gene therapy and small molecule product candidates.

We have acquired leading AAV gene vector technology and developed proprietary delivery methods which form the basis of our MYDICAR platform. In addition, using our proprietary, patented SERCA2 screening assay, we have developed a broad platform of novel, first-in-class, small molecule modulators of the SERCA2b enzyme, creating development opportunities for product candidates targeting diseases associated with endoplasmic reticulum, or ER, stress-related pathways, such as diabetes and neurodegenerative diseases.

The following figure illustrates the opportunities and approach we are taking to target SERCA2 deficiency states:

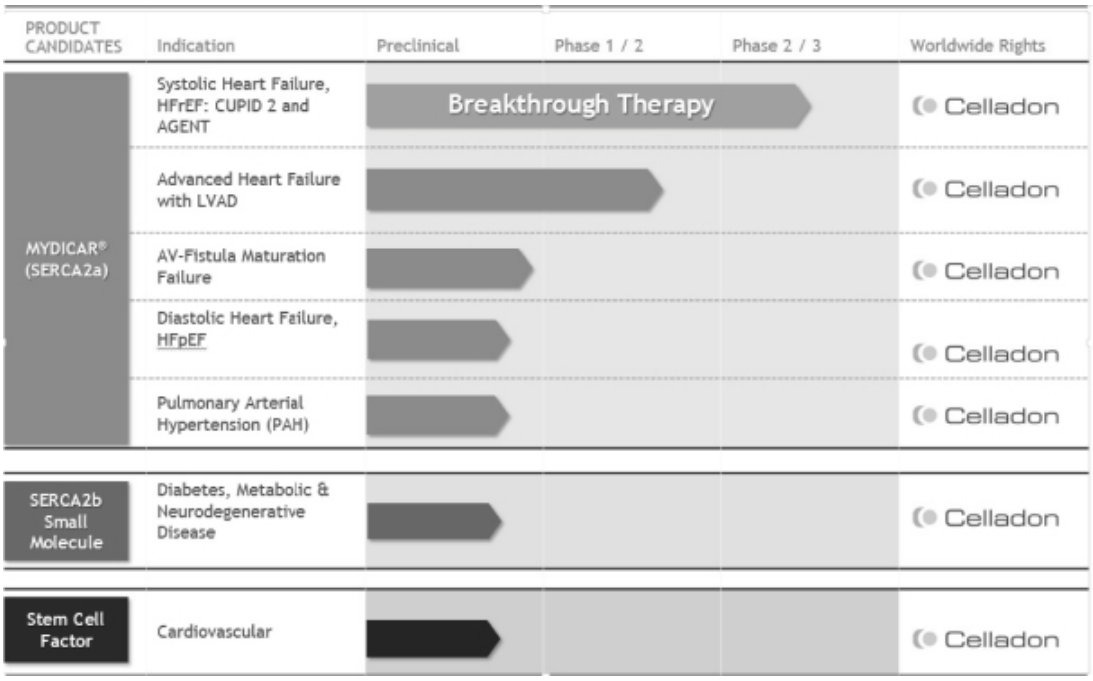


Our lead development program targets calcium dysregulation in the heart. Of the ions involved in the intricate workings of the heart, calcium is considered perhaps the most important. Proper calcium cycling enables the chambers of the heart to pump, or contract and relax, which causes blood to be propelled in and out of the heart. Calcium directly activates the myofilaments, which are threadlike structures in muscle fibers which cause contraction. Dysregulation of calcium is a central cause of heart failure due to both contractile (systolic) dysfunction, and relaxation (diastolic) dysfunction. One of the central causes of calcium dysregulation in heart failure is a deficiency in the level of SERCA2a enzymes in heart muscle cells. SERCA2a deficiencies are not limited to heart muscle cells, but are also present in blood vessel disorders such as AVF maturation failure and PAH.

Another focus of our research program relates to a different form of the SERCA2 enzyme, SERCA2b. Specifically, these enzymes control calcium movement in the ER in all human cells. SERCA2b enzyme levels become deficient when cells are stressed, and accumulate unfolded proteins in the ER, known as ER stress. There has been a proliferation of publications in scientific medical literature supporting the important role of ER stress in many diseases and conditions, including heart failure, diabetes and neurodegenerative diseases. We believe we are the industry leader in isolating small molecule modulators of the SERCA2b enzyme, which can correct underlying calcium dysregulation and ER stress. Our proprietary, novel, first-in-class, compounds have demonstrated activity in multiple preclinical models of diseases and conditions.

Our Product Pipeline

The following chart depicts key information regarding our development programs, their indications, and their current stage of development:



MYDICAR for Heart Failure

The Heart Failure Epidemic

Heart failure constitutes an important medical, social, and economic problem. Heart failure is a clinical condition in which the output of blood from the heart is insufficient to meet the metabolic demands of the body. In 2015, the American Heart Association estimated that there are nearly six million patients currently diagnosed with heart failure in the United States. The prevalence of heart failure is progressively increasing due to an aging population and increasing prevalence of major cardiovascular risk factors, including obesity and diabetes. Additional risk factors for heart failure include coronary heart disease, hypertension, alcoholism, drug abuse, exposure to toxins and infectious agents, pregnancy and congenital mutations. It is estimated that one in five adults at age 40 will develop heart failure during their remaining lifetime, and that approximately 250,000 to 500,000 patients in the United States are currently in the terminal phase of heart failure and have symptoms that cannot be effectively managed by existing optimized medical therapy. These patients suffer from disabling symptoms and often need hospitalization. The long-term prognosis associated with heart failure is worse than that associated with the majority of cancers, with approximate 50% mortality at five years following initial diagnosis. With over 280,000 heart failure-related deaths annually, we believe MYDICAR will provide a much needed therapeutic alternative for heart failure patients. We estimate that there are over 350,000 HFrEF patients in the United States who would be eligible for MYDICAR treatment upon launch, if approved.

Hospitalizations for heart failure are expensive and are particularly problematic, as the risk of death is increased with each recurrent heart failure-related hospitalization. There are one million primary heart failure-related hospitalizations annually in the United States alone. The estimated direct cost of heart failure in the United States in 2012 was $60.2 billion, half of which was related to repeated hospitalizations. By 2030, the total

cost of heart failure in the United States is projected to increase to $70 billion. The one- and six-month readmission rates after heart failure-related hospitalization are close to 25% and 50%, respectively. The Affordable Care Act recently established the "Hospital Readmissions Reduction Program," which requires Centers for Medicare & Medicaid Services to reduce payments to hospitals with excessive heart failure readmissions. As such, there is a growing pressure on hospitals to reduce readmissions for heart failure.

The pathologies resulting from heart failure are devastating. During heart failure progression, the heart steadily loses its ability to respond to increased metabolic demand, such as during intense physical activity. Patients suffer from increased shortness of breath in a progressive manner, and mild exercise soon exceeds the capacity of the heart to react to the increase in metabolic demand. Towards the end stage of the disease, the heart cannot pump enough blood to meet what the body needs even at rest. At this stage, fluids accumulate in the extremities or in the lungs, making the patient bedridden and unable to perform activities of daily living. In addition to constant shortness of breath, even minor deviation from a physical activity and diet restricted lifestyle can cause acute exacerbations, during which patients experience a drowning sensation and must be urgently hospitalized in intensive care or cardiac care units. Heart failure is classified in relation to the severity of the symptoms experienced by the patient. The most commonly used classification system, established by the New York Heart Association, or NYHA, is as follows:

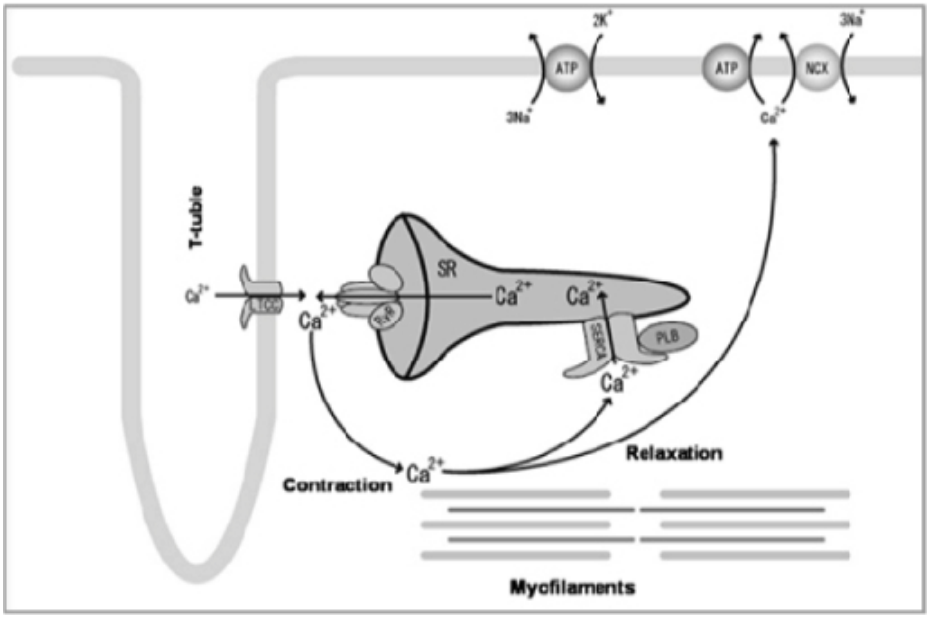
- Class I (mild): patients experience no or very mild symptoms with ordinary physical activity
- Class II (mild): patients experience fatigue and shortness of breath during moderate physical activity
- Class III (moderate): patients experience shortness of breath during even light physical activity
- Class IV (severe): patients are exhausted even at rest

The survival rate in each of these classes of heart failure is a function of the severity of the disease with the more advanced patients having poorer survival prognosis. Guideline-directed medical therapy for heart failure emphasizes angiotensin-converting enzyme, or ACE, inhibitors, angiotensin-2 receptor blockers if the patient is ACE intolerant, and beta blockers. There is recommendation for cardiac resynchronization therapy in certain patients. Implantable cardioverter-defibrillators, or ICDs, are used in patients at risk for sudden cardiac death. Despite these optimal guideline-directed therapies employing a wide range of pharmacologic, device, and surgical options, many patients deteriorate over time and develop advanced heart failure symptoms that cannot be effectively managed by existing optimized medical therapy. At the end stage of heart failure disease, current treatment options include heart transplant surgery or implantation of an LVAD. LVADs are battery operated mechanical circulatory devices used to partially or completely replace the function of the left ventricle of the heart for patients awaiting a heart transplant, or as a destination therapy for patients with NYHA Class IV heart failure who will never receive a heart transplant. Both of these end-stage treatment options require invasive open-chest surgery, include a host of complications such as lifetime immunosuppressive therapy in the case of transplant and risk of thrombosis and infection in the case of LVADs, and can cost in excess of $150,000. An estimated 1,500 patients per year in the United States have an LVAD implanted and an estimated 2,300 patients per year in the United States undergo heart transplant surgery.

Role of SERCA2a in Heart Failure

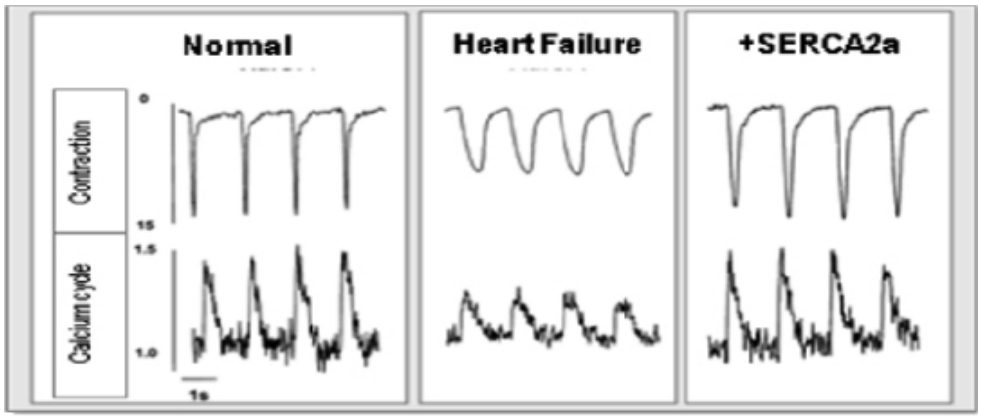
SERCA2a's role in heart failure was scientifically validated in the 1990s and immediately became a focus of pharmaceutical industry discovery efforts. However, due in part to ineffective screening technologies, SERCA2a proved to be an elusive target and to date no other company has been successful in targeting SERCA2a using traditional discovery methods.

Heart failure is characterized by abnormalities in the various steps of the heart muscle pumping process. Intracellular calcium movements in the heart are tightly regulated at various levels within the heart's cells. An organelle called the sarcoplasmic reticulum, or SR, plays an important role in orchestrating the movement of calcium during each contraction and relaxation. The cardiac cycle is illustrated in the figure below.



During contraction, calcium is released from the SR, activating the myofilaments leading to muscle contraction. During relaxation, the majority of calcium is sequestered back into the SR by the SERCA2a enzyme leading to muscle relaxation. It is modulated through normal physiology via a protein known as phospholamban (PLB in the figure above), increasing activity when we exercise and decreasing activity when we rest. In advanced heart failure, SERCA2a enzyme levels are abnormally low, so patients cannot effectively modulate SERCA2a activity which disrupts the normal calcium cycle. In turn, this negatively impacts normal contraction and relaxation and prevents the increase of cardiac output, even upon mild physical activity, such as walking or climbing stairs.

The figure below depicts *in vitro* studies of the contraction and relaxation and calcium cycle in normal human heart cells, in cells from patients with heart failure, and after correction of the SERCA2a deficiency in heart failure cells.



Even in end-stage human heart failure cardiac cells, correction of the SERCA2a deficiency is able to restore normal contractility, relaxation, and calcium cycling. This demonstrates the central importance of SERCA2a deficiency in heart failure, and the ability to reverse the abnormality in contraction and relaxation driving the pathogenesis of this serious medical condition.

Heart failure can be caused by a problem with cardiac contraction (systole), relaxation (diastole), or both. Ejection fraction, or EF, is the measurement used to describe the contractility of the heart. Approximately half of heart failure patients suffer from contractility abnormalities (EF less than 35%, HFrEF) while the other half suffer from relaxation (or diastolic) abnormalities (HFpEF). Both forms represent a significant unmet medical need. We are currently developing MYDICAR for HRrEF, but pending analysis of the CUPID 2 data, we also plan to develop MYDICAR to target the diastolic form of the disease (HFpEF). HFpEF is characterized by a stiff ventricle, which impairs relaxation of the heart between contractions. We believe MYDICAR can effectively treat HFpEF by correcting the SERCA2a deficiency and improving the ability of the heart to relax between contractions. Based on the Framingham Heart Study conducted by the National Heart, Lung and Blood Institute and Boston University, the five-year mortality rate for patients with HFpEF is 45–60%, which demonstrates the significant unmet need for effective treatments for this condition.

MYDICAR: Genetic Enzyme Replacement Therapy of SERCA2a Deficiency

Our lead product candidate, MYDICAR, uses genetic enzyme replacement therapy to correct the SERCA2a enzyme deficiency in heart failure patients that results in inadequate pumping of the heart. MYDICAR is delivered one-time, directly to the heart over a 10 minute period, in a routine outpatient procedure similar to an angiogram in a cardiac catheterization laboratory. MYDICAR has the potential to provide transformative disease-modifying effects with long-term benefits in heart failure patients with a single administration. We filed an investigational new drug application, or IND, in December 2006 for MYDICAR for the treatment of heart failure.

Gene therapy alters a person's deficient genetic material (encoded by deoxyribonucleic acid, or DNA). The altered genes, in turn, through a process called gene expression, can then produce the correct proteins and/or enzymes that were otherwise being produced improperly, or in the case of SERCA2 deficiency, at abnormally low levels. Gene therapy is accomplished through a process known as gene transfer, whereby a functional gene is delivered and incorporated into a patient's cells through a delivery system called a vector, which are most commonly based on naturally-occurring viruses that have been modified to take advantage of the virus' natural

ability to introduce genes into cells. However, unlike naturally-occurring viruses, which replicate following infection of a target cell and have the capacity to infect new cells, viral vectors are modified to be non-replicating by deleting that portion of the viral genome responsible for replication. We believe that the growing body of gene therapy-based clinical data and the establishment of regulatory guidelines to govern the development and approval of gene therapy products suggest that gene therapy is positioned to emerge as an important new therapeutic modality for patients with significant unmet medical need.

MYDICAR, or AAV1/SERCA2a, utilizes AAV1 to deliver the gene for the enzyme SERCA2a. AAV1/SERCA2a consists of an outer protein shell, called a capsid, and inner DNA genome that contains a gene for SERCA2a. In a treated patient, the capsid delivers the genome to the target cell, where the DNA will direct expression of the SERCA2a protein. Different strains of AAV, called serotypes, have slightly different capsids, which target the vector to different cell types. AAV vectors are particularly well suited for the treatment of heart failure because:

- AAV vectors are safe; most people are exposed to wild type AAV (serotype 2) during childhood, without developing any symptoms because AAV causes no disease. Regulatory authorities consider AAV vectors lower risk than other vectors commonly used in gene therapy, such as retroviruses or lentiviruses, because they present a low risk for inserting genetic material into the patient's chromosomes, which is known as insertional mutagenesis and may lead to cancer. This is because AAV DNA exists in the cell as a circle, or plasmid, outside the main chromosomal DNA.
- AAV vectors are less immunogenic than other viral vectors commonly used in gene therapy, which have caused inflammatory reactions in some patients.
- AAV1 results in a highly efficient delivery of genes into muscle cells so extremely small quantities can be administered directly to the heart to achieve a therapeutic effect; approximately 1/10,000 of a gram of AAV1 capsid protein is contained in a therapeutic dose. We have not observed any toxicities in our preclinical studies or clinical trials.
- AAV particles are small particles and pass freely through the blood vessel wall, bathing the heart muscle and providing broad distribution in the heart without the requirement for invasive or risky procedures. It is delivered directly to the heart over 10 minutes in a simple, one-time, outpatient procedure in a cardiac catheterization laboratory. Patients are awake under mild sedation, and outside of a small puncture in the groin or arm, feel no sensation as a catheter is advanced to the heart. Catheterization procedures like this are routine and are performed thousands of times a day around the globe for imaging the heart.
- AAV1, compared to other AAV serotypes such as AAV6/8/9, is well suited for expression in cardiac muscle after intracoronary infusion as it does not bind heparin sulfate. This allows passage of MYDICAR freely through the interstitial space which is important for perfusion of the interior portions of the myocardium. The lack of heparin sulfate binding also avoids the liver tropism of AAV serotypes such as AAV6. Furthermore, AAV1 but not AAV9, transduces endothelial cells, important for the vasodilator effects of MYDICAR. Finally, lack of heparin sulfate binding may reduce inflammatory immune response, as it likely directs uptake into human dendritic cells and activation of capsid specific T cells.

  Our AAV1 production and manufacturing technology has been developed with a focus on large-scale commercialization, and we believe we will be able to produce MYDICAR in large quantities to support our target markets.

After the AAV1/SERCA2a is infused in the arteries that feed the heart muscle, the AAV1 particle is taken up by the cells and results in expression of the normal SERCA2a human protein in the heart. This results in improved contractility, improved symptoms, and reductions in heart failure-related hospitalizations as demonstrated in our CUPID 1 trial.

Antibodies against AAV1 can block entry of MYDICAR into the target cells, and we have therefore developed a companion diagnostic to identify which patients do not have pre-existing NAbs against the AAV1 capsid proteins, and hence which patients are eligible for MYDICAR treatment. Even though the majority of the population has been exposed to wild type AAV (serotype 2), we believe approximately 40% of heart failure patients in the United States are AAV1 NAb negative and hence are eligible for MYDICAR treatment. In an effort to expand the population of patients who may be eligible for MYDICAR treatment for HFrEF, we are exploring whether plasma exchange can be used to remove AAV1 NAbs from the circulation in advance of MYDICAR administration, and have plans for a possible Phase 1/2 study of MYDICAR in advanced heart failure patients with HFrEF and pre-existing levels of NAbs against the AAV1 vector, who would undergo plasma exchange prior to administration of MYDICAR. Plans for this study are currently pending the outcome of the CUPID 2 trial and further discussions with the FDA.

MYDICAR is initially being developed to treat patients with HFrEF. Heart failure caused by HFrEF is characterized by a decreased contraction (EF less than 35%). We also plan to develop MYDICAR for additional indications, such as AVF maturation failure, and for the treatment of patients with advanced heart failure who are on an LVAD. Pending the outcome of the CUPID 2 trial, we expect to initiate a clinical trial in 2016 for the treatment of HFpEF, a condition caused by the inability of the heart to relax normally between contractions.

We estimate that there are over 350,000 HFrEF patients in the United States who would be eligible for MYDICAR treatment upon launch, if approved.

MYDICAR Previous Human Experience in HFrEF

We are the first company to enter clinical development with agents that selectively target this well-validated, key enzyme deficiency. In Phase 2a of the CUPID 1 trial, 39 patients with HFrEF were enrolled in a randomized-double-blind, placebo-controlled trial in which MYDICAR compared to placebo was found to be safe, reduced heart failure-related hospitalizations, improved patients' symptoms, quality of life and serum biomarkers, and improved key markers of cardiac function predictive of survival, such as end systolic volume, or ESV. The CUPID 1 trial included a single dose of MYDICAR with an on-study observation period of 12 months, plus a two-year long-term follow-up. Details are provided below, but an overall summary is as follows:

- MYDICAR was associated with benefit in clinical outcomes such as worsening heart failure, heart failure-related hospitalizations, need for LVAD implantation or heart transplant, or death.
- Benefit in clinical outcomes was supported by improvement in patients' heart failure symptoms, exercise tolerance, serum biomarkers, and cardiac function.
- High-dose MYDICAR (1 x 10^{13} DNase resistant particles) met the primary endpoint versus placebo at six months, and all positive trends were confirmed at 12 months.
- Benefit in preventing clinical events such as hospitalizations was confirmed at three years as well as a trend in improved survival.
- MYDICAR demonstrated an excellent safety profile.

CUPID 1, Phase 1 (CELL-001)

A total of 12 patients with heart failure (NYHA class III/IV) received a single intracoronary infusion of MYDICAR in an open-label dose-escalation trial in the United States. Administration of MYDICAR was on top of maximal optimized heart failure therapy. Doses administered ranged from 1.4 x 10^{11} to 1 x10^{13} DNase resistant particles, or DRP, per patient. The mode of administration was a 10-minute infusion into the coronary artery. MYDICAR demonstrated an excellent safety profile in this heart failure population, with no treatment related toxicities observed. Of the 12 patients who received MYDICAR, several demonstrated improvements from baseline to month six across a number of parameters important in heart failure, including symptomatic (NYHA and Minnesota Living with Heart Failure Questionnaire, five patients), functional (six-minute walk test

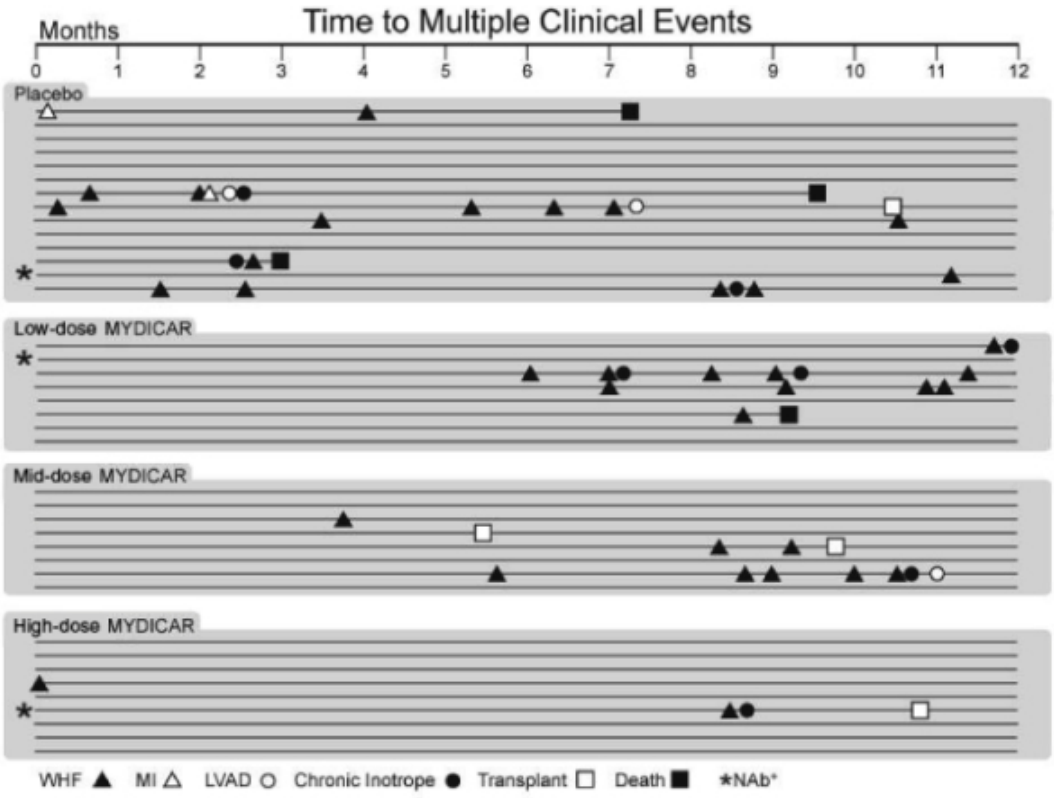
and peak maximum oxygen consumption, five patients), biomarker (N-terminal prohormone brain natriuretic peptide, or NT-ProBNP, two patients), and left-ventricular, or LV, function/remodeling (EF and ESV, six patients). Quantitative evidence of biological activity across a number of parameters important for assessing heart failure status could be detected in several patients without pre-existing NAbs in this open-label trial.

CUPID 1, Phase 2a (CELL-001)

The Phase 2a design was a randomized, double-blind, placebo-controlled trial in 39 patients who received one of three different doses of MYDICAR or placebo. Twenty-five patients received MYDICAR and 14 received placebo. The mode of administration was a one-time 10-minute infusion into the coronary arteries. All subjects had HFrEF (NYHA class III/IV). Treatment with either MYDICAR or placebo was on top of maximal optimized heart failure therapy. Seven efficacy parameters were assessed in four domains: symptoms (NYHA class and Minnesota Living With Heart Failure Questionnaire), functional status (six-minute walk test and peak maximum oxygen consumption), biomarker (NT-ProBNP), and LV function/remodeling (EF and ESV), plus clinical outcomes. The high-dose MYDICAR group versus placebo met the primary endpoint, which was demonstration of improvement across multiple domains without significant worsening in any domain. This combination of requirements resulted in a probability of success by chance alone (false-positive effect) of approximately 3%. The trial met the primary endpoint at six months (confirmed at 12 months) and demonstrated improvement or stabilization in the four efficacy domains.

The characteristics of recurrent clinical events and terminal events over the 12 months of the active observation period of the trial for Phase 2a portion of our CUPID 1 trial are illustrated in the figure below. Each line represents a single subject. Clinical events are depicted by symbols; a star at the beginning of a line represents subjects who were NAb positive at dosing. Patients who were AAV1 NAb positive at dosing had developed AAV1 NAbs during the period between their initial selection for participation in the trial and dosing, which in some cases, was as long as six months. We expect to continue to use our companion diagnostic to screen for AAV1 NAb status.

As can be seen from the figure below, despite maximal optimized background therapy, the clinical events (worsening heart failure, or WHF, myocardial infarction, or MI, LVAD implantation, use of chronic intravenous inotrope, heart transplant, or all-cause death) in the placebo group were substantial, underscoring the significant unmet need in this population, while in the high-dose MYDICAR group clinical events were limited. WHF was defined as signs and symptoms of heart failure requiring either hospitalization or treatment with intravenous diuretics, vasodilators or positive inotropes; mechanical fluid removal; or intra-aortic balloon pump.



Clinical Events in CELL-001 Phase 2a

In the low-dose (6×10^{11} DNase resistant particles) and mid-dose (3×10^{12} DNase resistant particles) groups, there was a delay to the onset of clinical events, and in the high-dose group, a significant reduction: the relative risk reductions, or hazard ratios, at 12 months for the high-dose MYDICAR group versus placebo for recurrent adjudicated clinical events of worsening heart failure (WHF) and myocardial infarction (MI) was 0.12, p=0.003 (where the p-value is the statistical probability of a result due to chance alone), representing a risk reduction of 88% for these important events with high-dose MYDICAR. At 36 months, the high-dose MYDICAR group versus placebo for recurrent adjudicated clinical events was 0.18, p=0.048, representing a risk reduction of 82% for these important clinical events with high-dose MYDICAR. The hazard ratios for recurrent clinical events at 12 months are summarized by treatment group in the table below.

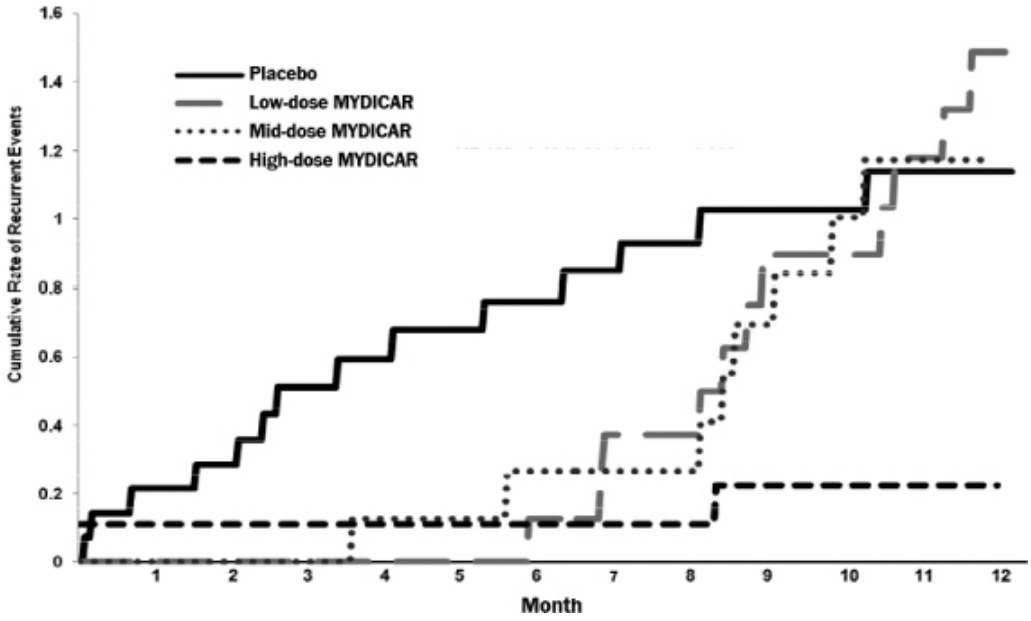
Time to Multiple Clinical Events Analysis at 12 Months

MYDICAR Dose vs. Placebo	Hazard Ratio (CI) for Recurrent Clinical Events (1)	Risk Reduction
Low-dose	0.40 (0.13, 1.21), p=0.11	60%
Mid-dose	0.44 (0.16, 1.24), p=0.12	54%
High-dose	0.12 (0.03, 0.49), p=0.003	88%

(1) Recurrent clinical events include WHF and MI.

In the low- and mid-dose groups, there was a delay to the onset of clinical events, and in the high-dose group a significant reduction. In the low- and mid-dose groups, we believe the dose was not sufficient to insert the SERCA2a gene in enough cells of the myocardium to generate a long-lasting improvement in contractility. We have confirmed this in biopsy samples (see “CUPID 1 (CELL-001) Long-term Follow-up” below), since MYDICAR vector DNA was only detected at long time points in cardiac biopsies in the high-dose patients, but not in biopsies from any other group. MYDICAR increases the presence of an enzyme called nitric oxide synthase in endothelial cells and this enables blood vessels to relax, thereby resulting in short-term increased blood flow. Our hypothesis for why the low- and mid-dose groups demonstrate a delay of the onset of clinical events which is not durable relates to the short-term increase in blood flow to the heart after MYDICAR therapy; higher doses are required to insert the gene deep into the cardiac muscle cells.

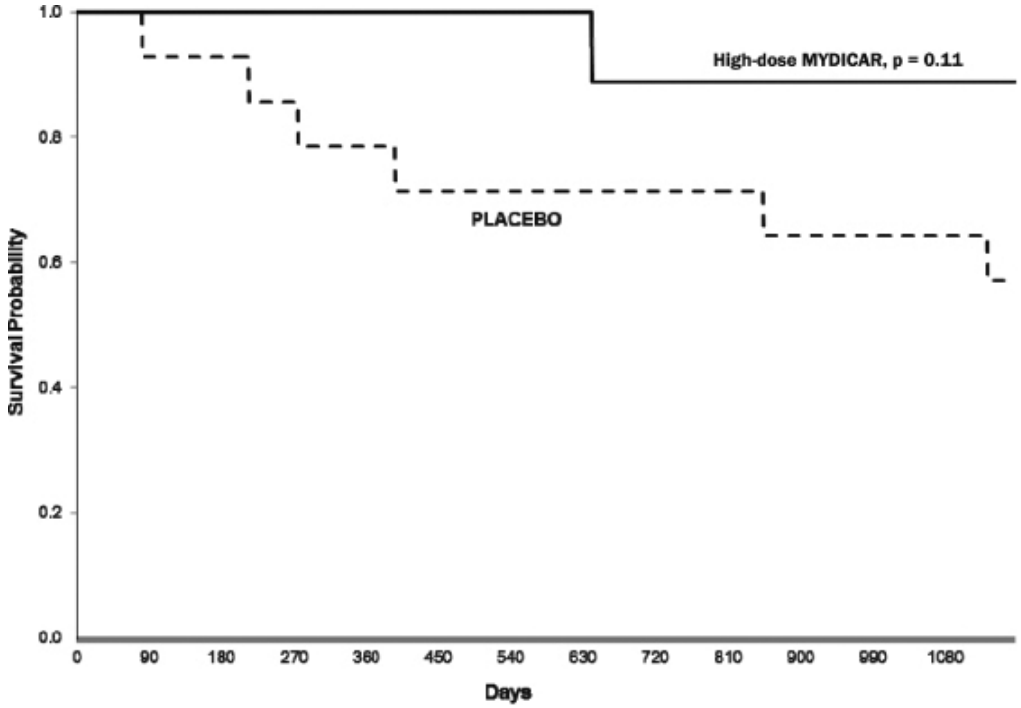
The frequency of clinical events (WHF and MI), are shown in the figure below.



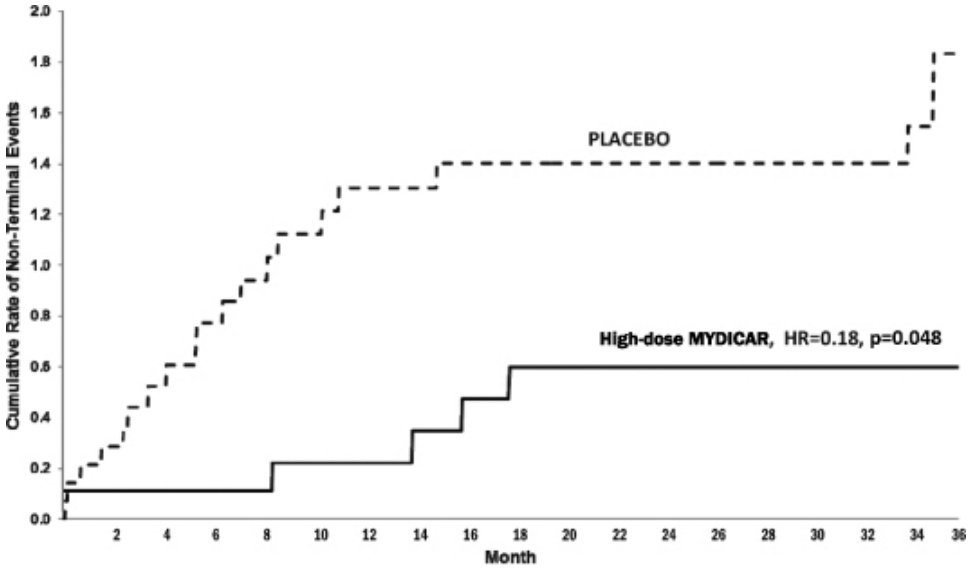
In addition to reducing the frequency of hospitalizations, the mean duration of heart failure-related hospitalizations over 12 months was substantially decreased (0.4 days versus 4.5 days; p = 0.05) on high-dose treatment versus placebo. Finally, there were no adverse safety findings.

CUPID 1 (CELL-001) Long-term Follow-up

The patients in the Phase 1 and Phase 2a portions of the CUPID 1 trial were followed for a total of three years. The following clinical events were tracked in all groups: WHF, LVAD implantation, heart transplantation, MI and all-cause death. At three years post-administration, there were 13 deaths: six in the placebo group, three in the low-dose group, three in the mid-dose group and one in the high-dose group (see the figure below for high-dose MYDICAR versus placebo).



Throughout the three years of follow-up, the number of clinical events was high in the placebo group and high but delayed in the low- and mid-dose groups. Few events occurred in the high-dose group where we found evidence of gene expression (the risk of pre-specified recurrent clinical events over three years of follow-up was reduced by 82% in the high-dose group compared to the placebo group, p=0.048). The figure below depicts cumulative clinical event rates over the three years of follow-up.



Finally, persistence of the AAV1/SERCA2a vector DNA in biopsy samples of the heart, in cases where heart tissue was made available, was demonstrated by a positive signal from quantitative polymerase chain reaction, or qPCR, testing in high-dose patients. We were only able to obtain heart tissue samples from patients who received an LVAD, cardiac transplant or who died in the hospital. The qPCR assay for AAV1/SERCA2a DNA has demonstrated persistence of the SERCA2a gene out to month 31 in the target tissue of one high-dose patient and to month 22 in another. A third high-dose patient demonstrated presence of vector DNA at month 23. All three patients with qPCR positive vector DNA results showing persistence of the AAV1/SERCA2a vector were in the high-dose group. The qPCR testing of available biopsy samples in patients from the placebo, low- and mid-dose groups did not demonstrate persistence of the AAV1/SERCA2a vector DNA.

Our CUPID 1 trial results demonstrated a favorable safety profile of MYDICAR. No increases in adverse events, disease-related events or laboratory abnormalities were observed in any of the MYDICAR-treated subjects compared to those receiving placebo over the three-year period. There was no indication of an increase in any new occurrences or exacerbation of pre-existing clinical conditions or prior disorders during long-term follow-up including malignancies, neurologic disorders, rheumatologic or other autoimmune disorders, hematologic disorders or other unexpected illnesses associated with MYDICAR administration.

Regulatory Overview and Current and Future Clinical Development of MYDICAR for HFrEF

The impact of MYDICAR on reduction of heart failure-related hospitalizations was an important finding from our CUPID 1 trial and current and future studies are designed to confirm these results and serve as the basis for potential regulatory approvals in the United States. Following completion of our CUPID 1 trial, we held an End-of-Phase 2 meeting with the FDA, as a result of which the FDA indicated that:

- data supported proceeding to a Phase 3 clinical trial with MYDICAR;
- our proposed safety database, which will include approximately 610 patients (one-half treated), may be acceptable if the safety profile is similar to the safety profile demonstrated in our CUPID 1 trial;

- time to recurrent heart failure-related hospitalizations is acceptable as the primary endpoint, pending details of the statistical analysis plan and further discussion with agency statisticians, which have since been accepted including their agreement that the method to be used for the primary analysis, a joint frailty model, demonstrates adequate control of type 1 error; and
- a single trial may be acceptable for a BLA submission assuming a highly statistically significant estimate of treatment effect on the primary endpoint and strong concordance between primary and secondary endpoint analyses.

We held a Type A SPA meeting with the FDA, as a result of which the FDA approved a 572-patient Phase 3 trial protocol under the SPA guidance and agreed that the design and planned analyses of this trial would be sufficient to provide data that, depending on the outcome, could support a license application submission. Pursuant to the SPA, we also obtained an agreement from the FDA that the primary efficacy endpoint of time to recurrent heart failure-related hospitalizations would be acceptable for a pivotal trial of MYDICAR, using an ITT population for the primary analysis. This endpoint includes multiple heart failure-related hospitalizations per patient, and "corrects" for within-subject correlation and informative censoring from terminal events. Other elements of the Phase 3 SPA protocol, including sample size, may be changed if agreed to in writing by both the FDA and us. In April 2014, the FDA granted Breakthrough Therapy designation to MYDICAR for reducing hospitalizations for heart failure in patients who test negative for AAV1 NAbs, are class III or IV heart failure patients under the New York Heart Association, or NYHA, classification system, and are not in immediate need of a left-ventricular assist device, or LVAD, or heart transplant.

The design of our CUPID 2 trial is similar to the Phase 3 SPA protocol in that it includes the same primary and secondary end points, and the same method of analysis. However, in CUPID 2 we will use an mITT approach for analysis of the primary and secondary endpoints. We have adopted an mITT approach for the CUPID 2 trial in order to more accurately reflect intent to treat in the CUPID 2 study. We also discussed with the FDA our proposed use of an mITT population for the primary and secondary endpoint analyses, which excludes clinical events which occurred in patients that did not receive MYDICAR or placebo, or which occurred prior to dosing. This represents less than 5% of the clinical events that would have been included in an intent to treat analysis. Results from both the mITT and ITT approaches will need to be consistent to demonstrate a treatment effect. We believe that this is the appropriate approach for the CUPID 2 trial based on Section 5.2.1 of ICH Guidance for Industry, E9 Statistical Principles for Clinical Trials (Section V.B.1. of the corresponding FDA Guidance for Industry E9 Statistical Principles for Clinical Trials, September 1998) which provides that in some situations, it may be reasonable to eliminate from the set of all randomized subjects any subject who took no trial medication. In these situations, the intention-to-treat principle would be preserved despite the exclusion of these patients provided that the decision of whether or not to begin treatment could not be influenced by knowledge of the assigned treatment.

In May 2012, we participated in European Scientific Advice Meetings with local authorities at the Paul Ehrlich Institute in Germany and the College ter Beoordeling van Geneesmiddelen, Medicines Evaluation Board in the Netherlands. Advice from these meetings was incorporated into the Clinical Trial Application for the CUPID 2 clinical trial. In November 2013 we met with the Scientific Advice Working Part of the EMA to obtain scientific advice regarding the overall development program and most expeditious approval route for MYDICAR and advice from this meeting was incorporated into the development program. In November 2013, the EMA indicated that if MYDICAR demonstrates a substantial and highly significant treatment effect in the advanced heart failure population, a safety database of approximately 205-230 MYDICAR-treated subjects may be sufficient for a safety assessment of an MAA for MYDICAR for the treatment of HFrEF. Recently the EMA has indicated that they are not satisfied that adequate control of type 1 error (false positive rate) by the joint frailty model to be used for the primary efficacy analysis had been demonstrated. In addition, the EMA indicated that for this study to be considered as a single pivotal trial, a more extreme level of statistical significance than 5% would be required. We believe that our extensive simulation studies have demonstrated that when there are correlated recurrent events in the presence of terminal events that are also correlated with the recurrent events,

the joint frailty model provides unbiased treatment effect estimates, high power to detect treatment effect, and strong control of the false-positive (type-1 error) rate. We have included the traditional time-to-first-event analyses as a sensitivity analysis in the Statistical Analysis Plan for CUPID 2. After we un-blind the data from CUPID 2, we intend to continue discussions with the EMA regarding the use of the joint frailty statistical model for the primary analysis.

MYDICAR for HFrEF

CUPID 2 Trial (CELL-004)

The primary objective of our ongoing CUPID 2 trial is to determine the efficacy of a single, one-time, intracoronary infusion of high-dose MYDICAR compared to placebo, in conjunction with maximal optimized heart failure therapy, in reducing the frequency of and/or delaying heart failure-related hospitalizations in patients with HFrEF (EF less than 35%) who are at increased risk of terminal events based on elevated levels of NT ProBNP or a recent heart failure-related hospitalization.

The population is adult patients, 18 to 80 years of age, with NYHA class II/III/IV symptoms of heart failure due to ischemic or non-ischemic cardiomyopathy, and who, despite maximal optimized heart failure therapy regimens, are at high risk of heart failure-related hospitalizations. A total of 250 patients (N= ~125 per treatment arm) were randomized for the purpose of obtaining at least 186 adjudicated heart failure-related hospitalizations.

Patients were randomized in parallel to 1 x 10^{13} DRP MYDICAR or placebo in a 1:1 ratio. The trial is being conducted at approximately 53 sites in the United States, Denmark, Sweden, Germany, Poland, Belgium, the Netherlands, the United Kingdom, Israel and Hungary, with randomization stratified by country.

Potential trial participants were prescreened for the presence of NAbs against AAV1 using our companion diagnostic. Those who tested negative for AAV1 NAbs underwent further screening tests and procedures to determine eligibility prior to randomization and enrollment into the trial. Those who tested positive for AAV1 NAbs were excluded from the trial. The primary data analysis cutoff was reached in February 2015 when all patients completed the full 12-month active observation period and at least 186 adjudicated heart failure-related hospitalizations had occurred.

In CUPID 2, we enrolled advanced heart failure patients at high-risk for serious adverse events and death. An independent data monitoring committee, or DMC, responsible for monitoring safety of the trial, has met five times. In all five meetings the DMC recommended that CUPID 2 proceed with its trial protocol as planned. Because CUPID 2 is a clinical outcomes trial, all clinical events are reviewed by both the un-blinded DMC and by an independent blinded Clinical Endpoint Committee, or CEC. The primary endpoint will be assessed at one year and all patients will be followed for a total of five years.

In CUPID 2 the endpoints were chosen to fully capture disease burden and to gain efficiency by including all terminal events (e.g. all-cause death, heart transplants and LVAD implantation) in the analyses. There are many statistical methods for the analysis of recurrent events; however, the joint frailty model addresses the limitations of other approaches, as it accounts for the correlation between the recurrent event process and the terminal event process (informative censoring).

The primary efficacy endpoint is time-to-recurrent heart failure-related hospitalizations in the presence of terminal events at the time of primary analysis data cutoff. In the primary endpoint analysis the treatment effect estimate (hazard ratio for recurrent heart failure-related hospitalizations for MYDICAR versus placebo adjusted for correlated terminal events), will be calculated using the joint frailty model. The secondary endpoint is time-to-first terminal event (all-cause death, heart transplant or LVAD implantation). Additional exploratory endpoints include Kansas City Cardiomyopathy Questionnaire (quality of life) and six-minute walk test (exercise capacity). NYHA class will be descriptively summarized by time point for each treatment group.

The sample size for our CUPID 2 trial is based on Monte Carlo simulations so that approximately 250 patients with an estimated total of 186 heart failure-related hospitalizations should provide at least 83% power, at the 0.05 two-sided significance level, to detect at least a 45% risk reduction (hazard ratio of 0.55) based on time-to-recurrent heart failure-related hospitalizations in the presence of the terminal events. The assumed magnitude of treatment effect is based on the data from published studies in heart failure patients and a conservative estimate of the anticipated magnitude of effect of MYDICAR based on 12-month results from CUPID 1 that showed an 88% reduction in recurrent clinical events adjusted for correlated terminal events with high-dose MYDICAR compared to placebo. We completed enrollment of this trial in February 2014, reached the primary analysis data cutoff in February 2015, and expect to un-blind the data and announce results in late April 2015.

CUPID 3 Trial (CELL-003)

We are currently preparing for another international trial of MYDICAR. This trial can either serve as an additional trial for regulatory approval or to further characterize MYDICAR's therapeutic profile.We anticipate that the design of this double-blind, randomized, placebo-controlled trial will be similar to CUPID 2. Potential trial participants will be prescreened for the presence of NAbs against AAV1 using our companion diagnostic. Those who test negative for AAV1 NAbs will undergo further screening tests and procedures to determine eligibility prior to randomization and enrollment into the trial. Those who test positive for AAV1 NAbs will be excluded from the trial. Data analyses will be performed when all patients have completed the full 12-month active observation period and the requisite number of adjudicated recurrent events have occurred.

In CUPID 3, we will enroll advanced heart failure patients, with HFrEF who are at high-risk for serious adverse events and death. An independent DMC will be responsible for the routine monitoring of the safety of the trial. Because CUPID 3 will be a clinical outcomes trial, all clinical events will be reviewed by both the un-blinded DMC and by an independent blinded CEC. The primary endpoint will be assessed at one year and patients will be followed for a total of five years.

The sample size for our CUPID 3 trial will be finalized following analysis of CUPID 2.

AGENT-HF Trial (AAV1-CMV-SERCA2a Gene Therapy Trial in Heart Failure)

This trial is an investigator initiated clinical trial which commenced screening in December 2013. The trial is partially funded by the French government and sponsored by Assistance Publique—Hôpitaux de Paris. We are providing investigational medicinal product and some financial support.

The primary objective of the AGENT-HF Trial is to determine whether a one-time treatment with MYDICAR leads to reverse remodeling of the heart. In patients with heart failure, the size, shape, structure and physiology of their heart changes over time, and these changes that lead to a progressive decline in left ventricular function are referred to as remodeling. In reverse remodeling, there would be changes back to the more normal, healthier state of the heart along with an improvement in the functioning of the heart. This trial is expected to enroll approximately 44 heart failure patients in France with half receiving MYDICAR and the other half placebo. The primary endpoint at six months will be change, compared to baseline, in left ventricular end systolic volume as measured by cardiac computed tomography. As of March 11, 2015, there were 10 subjects enrolled in this study.

CELL-005 AAV1 NAb Positive Trial

The primary objective of the AAV1 NAb positive trial is to determine the safety of a single one-time intracoronary infusion of high-dose MYDICAR in patients who test positive for AAV1 NAbs. The FDA has required this safety study as a condition to the submission of a BLA, to cover the possibility that MYDICAR may be used off-label in AAV1 NAb positive patients. In addition, the trial would explore the potential level of activity of MYDICAR in AAV1 NAb positive patients, although the trial would not be of sufficient size to detect

statistical differences in the response in patients who test positive for AAV1 NAbs versus those who test negative. The patient population would be similar to the target patient population in our CUPID 2 trial and would be approximately 60 patients. The study design would be a Phase 2, randomized, double-blinded, parallel study. Patients would be stratified by baseline AAV1 NAb titer—either negative/equivocal or positive (³1:2)—and randomized in parallel, in a 2:1 ratio, to either MYDICAR or placebo. The primary endpoint after all subjects had been followed for at least six months would be safety as measured by the incidence and severity of adverse events, including all-cause mortality and heart failure-related hospitalizations. The percentage of subjects experiencing an event would be calculated for survivors and for all patients enrolled. Frequency, type and duration of cardiovascular hospitalizations would also be analyzed. The CEC would classify all deaths and hospitalizations, distinguishing between the primary cause and immediate underlying cause of death or hospitalization. The following activity/efficacy variables would be summarized descriptively by treatment group as the trial is not powered to detect a statistical significance in any of the variables: left ventricular end systolic volume, distance walked during the six-minute walk test, NT-proBNP levels, NYHA classification, and quality of life assessed by the Kansas City Cardiomyopathy Questionnaire. We currently plan to initiate this trial in 2015 or 2016.

CELL-006 Viral Shedding Trial

The viral shedding trial is required as part of the environmental risk assessment that must be included in a marketing application to regulatory authorities, both in the United States and in Europe. In this open-label trial, approximately 10 to 20 patients with heart failure (the same target patient population as our CUPID 2 trial and our AAV1 NAb positive trial) would be treated with MYDICAR and followed until they have three consecutive bodily fluid samples that are negative for presence of the SERCA2a gene, as assessed by qPCR. The patients would continue to be followed for safety for up to two years to add to the overall MYDICAR safety database. With the information from this trial, the marketing application would have information on how long treated patients would be excreting MYDICAR into the environment, thereby potentially spreading the virus to family members, health care workers and the public. We currently plan to conduct this trial in 2016.

CELL-008 Plasma Exchange Pilot Study

After we un-blind the CUPID 2 data, we plan to discuss with the FDA the opportunity to potentially conduct a pilot, 24 patient, Phase 1/2 study of MYDICAR in advanced HFrEF patients who have been previously excluded from MYDICAR studies in this indication due to pre-existing levels of NAbs against the AAV1 vector.

CELL-009 High Dose Trial

The primary objective of the Phase 1/2 CELL-009 study is to characterize the safety profile of MYDICAR at a 2.5-fold higher dose than previously studied in order to further define MYDICAR's therapeutic index. In addition, this 36 patient study will explore the preliminary activity and efficacy using a composite outcome in five domains: symptomatic (NYHA class and quality of life), functional (distance walked in six minutes), blood biomarker of disease (NT-proBNP), left ventricular remodeling (change in left ventricular end systolic volume) and clinical outcome (recurrent and terminal events). The patient population will be adult patients with advanced chronic stable NYHA class III or IV HFrEF who are showing signs of progression despite optimal drug and device therapy. The study design consists of a lead-in Phase 1 open-label study of three to six subjects immediately followed by a Phase 2 randomized, double-blinded, placebo-controlled study. The primary safety analysis, performed after all subjects in the Phase 2 portion of the study complete at least six months of observation, will include the following safety endpoints: proportion of subjects who complete the study; adverse event incidence, severity and relationship to MYDICAR; concomitant medical use and changes in heart failure-related medications; incidence and event rates of hospitalizations, ambulatory worsening heart failure, myocardial infarction (heart attack), stroke, mechanical circulatory support device implantation, heart transplant and death; changes from baseline in laboratory tests, 12-lead ECG and physical examination; and changes from

baseline in implantable cardioverter defibrillator interrogation parameters. This study has been initiated, with patient recruitment and enrollment expected to commence during the second quarter of 2015.

Preclinical Studies of MYDICAR in HFrEF

Preclinical studies have shown that, after administration of an AAV vector, the plasmids containing the vector DNA are cleared from the blood and tissues via the mononuclear phagocyte system in liver, spleen and lymph nodes, and lungs. After intracoronary delivery, AAV particles which are not taken up in cardiac tissues are first passed through to the lung via the coronary sinus, making this the first pass organ. Stable, long-term presence of viral DNA, SERCA2a protein, and vector-derived SERCA2a mRNA have been demonstrated in cardiac tissue of normal rats for up to one year following a single administration of MYDICAR.

Gene transfer of SERCA2a is associated with improved cardiac function in various rodent models of heart failure. Improved heart function and enhanced expression of SERCA2 have also been demonstrated in an ovine (sheep) pacing-induced heart failure model with MYDICAR. SERCA2 gene transfer has also been associated with restoration of SERCA2a expression and improved heart function in both a dog-pacing heart failure model and in a chronic myocardial ischemia-induced heart failure model in mini-pigs. Beyond the effects on enhancing contractility, SERCA2a gene transfer has been shown in preclinical studies to restore the energetic state of the heart (both in terms of energy supply and utilization), to decrease arrhythmias, and enhance blood flow to the heart through expression in endothelial cells.

Several studies we have sponsored have established pharmacologic activity for MYDICAR gene transfer in animals with heart failure, with data demonstrating restored SERCA2a expression and stabilization/improvement in heart function. The pharmacology study was conducted in the porcine (pig) mitral regurgitation, or MR, heart failure model. MR induces reduced myocardial contractility, elevated B-type natriuretic peptide, or BNP, levels and other signs and markers which are virtually identical to those associated with the human disease, including a decrease in SERCA2a expression. MYDICAR-treated animals demonstrated significant improvements in the heart's ability to contract and relax and improved ventricular volumes. In these studies, there was an absolute increase of 16% in median EF in MYDICAR-treated animals as compared to control animals. ESV increased in the control group by a median of 16 milliliters, or a median relative increase of 35%, an indication of decreased contractility and cardiac enlargement, compared with the MYDICAR group, which showed a tendency to decrease LV ESV by a median of 9.9 milliliters (a median decrease of 14%). In humans, a reduction in ESV of 10% signifies clinically relevant reverse remodeling, which is a strong predictor of lower long-term mortality and heart failure clinical events. Treated animals also had lower BNP levels post-dosing.

We have also sponsored two safety toxicology and biodistribution studies, both in normal mini-pigs. Both were three-month studies simulating the clinical administration procedure for MYDICAR or placebo with 5, 30 and 90 day sacrifice time points. Doses of up to three times the human dose on a weight-adjusted basis were administered. No mortalities were observed in either study and treatment with MYDICAR was not associated with any signs of toxicity or effects on body weight, sperm motility, clinical pathology, gross pathology, clinical chemistry parameters, organ weights or histopathology. No significant effects were observed on cardiovascular parameters, including electrocardiographic intervals. There were no test article-related observations during the necropsies. Mild increases in troponin I were observed in eight out of a total of 36 MYDICAR-treated animals in the first study, barely above upper limits of normal for humans. These increases were not considered to be related to MYDICAR or biologically significant and were not observed in the second study. No treatment related changes in troponin I values were observed across the other large animal pharmacology studies.

MYDICAR in Additional Indications

Beyond our proposed lead indication of HFrEF, we are also developing MYDICAR for additional indications including enhancement of AVF maturation, HFpEF, pulmonary arterial hypertension, or PAH, and treatment of patients with advanced heart failure who are on an LVAD. Each of these conditions is characterized

by a SERCA2a deficiency, and MYDICAR has demonstrated disease-modifying capability in preclinical models of these diseases. We are currently engaged in preclinical research regarding MYDICAR for the treatment of AVF maturation and HFpEF, and plan to initiate human clinical trials in these indications in 2016 if supported by the CUPID 2 results. The broad potential of MYDICAR in multiple indications presents opportunities to maximize the value of our development programs for indications that are poorly managed by existing treatment options.

MYDICAR in Arteriovenous Fistula Maturation Failure (SERCA2a-AVF)

Currently, over 500,000 Americans have end-stage renal disease requiring dialysis. An arteriovenous fistula, or AVF, which is a surgically created connection between an artery and a vein in the arm of the patient, has proven to be the most durable, least complicated, and therefore preferred mode of vascular access for hemodialysis. The access that is created is routinely used for hemodialysis two to five times per week. Approximately 100,000 fistulae are placed yearly in the United States. However, a clinical problem that has resulted from this practice is that, following surgery to create the fistula, approximately 50% of the fistulae fail to mature to a usable state, and as many as 25% of hospital admissions in the dialysis population have been attributed to vascular access problems, including fistula malfunction and thrombosis.

Role of SERCA2a in Arteriovenous Fistula Maturation Failure

We believe MYDICAR has the ability to provide patients with end-stage renal disease a reliable and durable vascular access site for hemodialysis. The role of SERCA2a in normal and diseased blood vessel biology has been extensively studied. Maturation failure of an AVF has been attributed to rapid proliferation of VSMC, resulting in vascular occlusion. The histological lesion that appears to be associated with early AVF failure is referred to as neointimal hyperplasia, comprising VSMC, myofibroblasts and endothelial cells within microvessels. In the setting of early AVF failure, both aggressive neointimal hyperplasia and adverse vascular remodeling (vasoconstriction or an inability to dilate adequately) plays a role. In particular, the combination of early and aggressive neointimal hyperplasia together with adverse vascular remodeling results in aggressive early stenosis. The biology of SERCA2a in both VSMC and endothelial cells provides a unique opportunity to potentially positively impact these pathological processes:

- Proliferation of VSMC is associated in the rat, rabbit, and human with loss of SERCA2a expression and is thought to be the dominant cell type driving neointimal hyperplasia. SERCA2a gene transfer inhibits in vitro VSMC proliferation and prevents neointimal thickening in a rat carotid-injury model and prevented in-stent restenosis using an ex vivo model of human left internal mammary artery intimal thickening.
- In endothelial cells, SERCA2a modulates endothelial nitric oxide synthase, or eNOS, expression and activity. This enzyme produces nitric oxide, which dilates blood vessels. In a swine model of heart failure, coronary artery blood flow was decreased significantly, and MYDICAR rescued blood flow to levels observed in normal animals. In human artery endothelial cells, SERCA2a overexpression increased eNOS expression, phosphorylation, promoter activity and cellular Ca^{2+} storage capacity. Thus, SERCA2a gene transfer increases eNOS expression and activity by modulating calcium homeostasis, resulting in dilated blood vessels and improved blood flow.
- MYDICAR was tested in a pharmacology safety study in a swine model of vascular injury. MYDICAR-treated animals demonstrated reduced neointimal hyperplasia and less stenosis as compared to the control animals.

The purpose of the proposed SERCA2a AVF trial will be to determine if MYDICAR, when applied to a limited segment of blood vessel during surgery to create an AVF, is safe, dilates the blood vessel, helps keep vessels open and improves the long-term function of the AVF. We are currently conducting preclinical work to

support an IND for this potential new indication. Pending completion of this preclinical work and agreement by the FDA, we intend to conduct a Phase 2a trial with MYDICAR in end-stage renal disease patients undergoing surgery for AVF creation.

MYDICAR—LVAD Trial Investigation of the Safety and Feasibility of AAV1/SERCA2a Gene Transfer in Patients with Heart Failure and an LVAD

This ongoing trial is partially funded by the British Heart Foundation and is sponsored by Imperial College London. We are providing investigational medicinal product and some financial support. It is not a required trial by any regulatory authorities; however, it could potentially serve as a proof-of-concept trial to support the use of MYDICAR to wean patients off of an LVAD. The use of these devices present a host of risk factors for the patient, such as increased risk of thrombosis and infections, and these devices do not last for long periods of time. Given that the circulatory system of a patient with an LVAD is dependent on these devices, device failure usually translates to a catastrophic event for the patient. The primary objectives of the SERCA2a-LVAD trial are to determine (1) the safety and feasibility of using MYDICAR to treat heart failure patients who have an LVAD, (2) how well MYDICAR delivers the gene for SERCA2a to heart cells and (3) what impact circulating NAbs to AAV1 have on the ability of MYDICAR to deliver the SERCA2a gene to heart muscle cells. This trial is expected to enroll approximately 24 patients in the United Kingdom with varying levels of circulating NAbs to AAV1, 16 of whom will be treated with MYDICAR and eight with placebo. Six months post-treatment, all patients will undergo a heart biopsy for collection of tissue to determine the presence of the SERCA2a gene. In addition, safety data and changes in LV function will be collected and analyzed. As of March 11, 2015, there were five subjects enrolled in this study.

MYDICAR for HFpEF

As in HFrEF, a consistent finding in HFpEF is a decrease in the expression of SERCA2a—a change that is seen in most animal models of heart failure and in human hearts with diastolic dysfunction. In preclinical studies, overexpressing SERCA2a using gene therapy in streptozotocin-treated transgenic mice demonstrated that increasing SERCA2a could improve diastolic function. In human cardiomyocytes isolated from the left ventricle of patients with end-stage heart failure, SERCA2a levels were correlated with improved diastolic function. We have also evaluated MYDICAR in another preclinical study in a rat model for spontaneous non-insulin-dependent type 2 diabetes mellitus, which is characterized by diastolic dysfunction and associated with abnormal calcium levels and decrease in SERCA2a expression. In this study, SERCA2a gene transfer restored diastolic function to normal. These data showed that SERCA2a overexpression may be used as a therapeutic strategy for the treatment of this disease.

SERCA2a gene transfer has also been demonstrated to improve diastolic cardiac function in aged animals. In preclinical studies, cardiac SERCA2a protein and ATPase activity were significantly decreased in elderly rat hearts compared with adult rats and were restored to adult levels after SERCA2a gene transfer. Diastolic function parameters, which were adversely affected in elderly rat hearts, were restored by overexpression of SERCA2a, supporting the hypothesis that decreased SERCA2a contributes to the functional abnormalities observed in elderly hearts and demonstrating that targeting SERCA2a in the elderly heart may lead to improved diastolic cardiac function.

The MYDICAR-HFpEF trial would be our pilot clinical trial for the treatment of HFpEF, which comprises approximately half of all heart failure cases. We anticipate that the existing data we have generated for our proposed HFrEF indication would allow us to launch directly into a Phase 1/2 trial. Pending the outcome of CUPID 2, we expect to initiate clinical studies for this proposed indication in 2016.

MYDICAR—Pulmonary arterial hypertension (PAH)

Pulmonary arterial hypertension (PAH) is a syndrome defined by complex vascular disease and an increase in pulmonary artery pressure that results in heart failure and premature death in affected patients. The lungs in PAH patients are characterized by changes in the structure of small pulmonary arteries reducing artery diameter and impeding flow through them. This induces a rise in pulmonary vascular resistance, increasing the stress on the right side of the heart which progresses to an enlarged and impaired right ventricle, a decrease in cardiac output, heart failure, and death.

In both animal models and samples from human patients with PAH, pulmonary tissue shows reduced expression of SERCA2a, an enzyme critical for proper pumping of calcium in calcium compartments within the vascular smooth muscle cells. When these vascular smooth muscle cells begin to proliferate under pathological conditions, SERCA2a expression is lost. In addition, overexpression of SERCA2a in endothelial cells results in increased nitric oxide release and vasodilation of the arteries. In PAH animal studies, an exogenous SERCA2a gene via gene therapy using MYDICAR has introduced a healthy SERCA2a gene into pulmonary cells using a nebulizer-like inhalation device. MYDICAR has demonstrated evidence of PAH reversal in rat and pig models of the disease. In a pig model of PAH, a single one-time dose inhaled tracheal delivery of MYDICAR, at two months, resulted in significant decrease in mean pulmonary arterial pressure, reduction in pulmonary vascular resistance, improvement in RV function, reduction in the fulton index (size of right ventricle/size of left ventricle + Septum) and reverse remodeling of the pulmonary arteries. We are currently conducting a follow-up study in a pig model, evaluating the durability of a single dose of MYDICAR at six months.

Small Molecule Program

We have initiated several pre-clinical studies with our novel, first in class, small molecule modulators of SERCA enzymes including for the treatment of diabetes and neurodegenerative diseases. We believe these compounds may correct underlying calcium dysregulation and ER stress which are implicated in many disease states.

Membrane-bound form of Stem Cell Factor (mSCF)

Following our acquisition in July 2014 of worldwide rights to gene therapy applications for the membrane-bound form of Stem Cell Factor for treatment of cardiac ischemia, we are pursuing an additional gene therapy product opportunity in mSCF for the treatment of cardiac ischemic damage. Stem cell research to date has demonstrated potential to treat heart failure, pulmonary disease, type 1 diabetes mellitus, Parkinson's disease, Huntington's disease, Celiac Disease, muscle damage, along with many others. mSCF is a powerful growth signal for c-*kit*$^{+}$ stem cells, and is the ligand for the tyrosine kinase receptor c-*kit*. mSCF induces c-kit+ stem/progenitor cell expansion *in situ*, as well as cardiomyocyte proliferation, which may represent a new therapeutic strategy to reverse adverse remodeling after cardiac injury. In a preclinical setting, mSCF has demonstrated potential improvements in cardiac function and survival following a myocardial infarction. Specifically, these data suggest mSCF gene therapy promoted a regenerative response characterized by an enhancement in cardiac hemodynamic function; an improvement in survival; a reduction in fibrosis, infarct size and apoptosis; an increase in cardiac c-kit+ progenitor cells recruitment to the injured area; an increase in cardiomyocyte cell-cycle activation; and Wnt/ß-catenin pathway induction. To date, however, cell therapy for tissue repair has been hampered by the complexities of using cells as products from a delivery, manufacturing, and regulatory perspective.

Our approach with mSCF gene therapy is to recruit and expand *resident* stem cells, thereby harnessing advances in gene therapy technologies and also expanding the application to those in which cardiac stem cells have shown promise in clinical and preclinical testing. We are currently generating gene therapy vectors in support of potentially conducting a future clinical trial in patients who have suffered cardiac damage, as well as exploration of other potential applications. Preclinical studies using AAV mediated vector delivery of the mSCF gene for promoting cardiac regeneration post myocardial infarction are planned for 2015.

Sales and Marketing

We currently have full worldwide commercial rights to all of our development programs. We believe we can maximize the value of our company by retaining substantial commercialization rights to our product candidates and, where appropriate, entering into partnerships for specific therapeutic indications and/or geographic territories.

Our current strategy is to market MYDICAR for all potential heart failure indications using a dedicated specialty sales force calling on selected cardiologists, heart failure specialists and other health care providers. These physicians are typically affiliated with leading hospitals and medical centers and we believe that they tend to have well-established referral networks with supporting interventional cardiologists and cardiac catheterization laboratories. We believe they represent a concentrated customer base suitable to a specialty sales model. We believe that MYDICAR would be adopted first by high-volume key-opinion-leader hospitals and medical centers, and progressively by a broader segment of the market. Cardiologists, heart failure specialists, and interventional cardiologists, have a history of early adoption of innovative products and technologies, in part because the rate of innovation in this sector has been sustained, and in part because of the large unmet need that their patients exhibit.

We therefore believe that a commercial strategy involving a progressive build out of commercial infrastructure in the United States covering key prescribers and centers of excellence is one that we can realistically pursue. Our commercialization strategy for MYDICAR in different geographies and indications beyond heart failure will continue to be evaluated and may involve strategic partners.

Manufacturing of MYDICAR (AAV1/SERCA2a)

AAV has many characteristics that facilitate large scale manufacturing and distribution, when exploited effectively. We believe that our significant investment in AAV1/SERCA2a process development and analytical characterization has paid off in an inherently scalable, proprietary manufacturing process that is capable of supplying a global market as large as heart failure with a gene therapy product.

The technology includes a coordinated design of the AAV1/SERCA2a vector genome (the vector DNA) and the production system. AAV vectors are made “gutless,” meaning that they do not contain viral genes. Only the two small non-coding elements from the parent virus are needed for replicating and packaging the vector DNA during production, which can be provided separately. The genome was also designed to be very close to the size of the parent AAV genome, to optimally fit within the AAV capsid.

Our state of the art manufacturing process for AAV1/SERCA2a was developed based on proven industrial cell culture methodologies. Like many of the manufacturers of recombinant monoclonal antibodies or proteins, we use cell-suspension based culturing techniques and intend to use stirred tank bioreactors for large scale cell culture and production. Our planned commercial production scale is 2,000 liters, which is one-tenth the volume of the largest industrial production vessels, so our anticipated production scale is well within the limits of the technology. We selected stirred tank production bioreactor technology as our production system because it has been the workhorse for recombinant protein production for more than 20 years. For purification of AAV1/SERCA2a, we use industrial chromatography columns and resins, and filtration technology common to the biopharmaceutical industry. We believe these materials and equipment are common for manufacturing of FDA approved biological products.

Our Approach for Producing AAV1/SERCA2a

By specifically creating a cell line for the manufacture of AAV1/SERCA2a that has the necessary components stably integrated into the cell line, we have created a production process similar to other industrial scale processes used to treat large market disease indications.

We use standard cell culture techniques and standard equipment in production and purification found in industrial scale cell culture drug manufacturing. All media used for cell growth and production are free of animal serum and of high risk animal-derived components. To induce production of AAV1/SERCA2a, the cells are infected with a highly characterized batch of adenovirus. AAV viruses in nature and AAV vectors are not capable of replicating on their own and require a helper virus, such as adenovirus, to initiate replication. The purification process was designed to yield a high purity AAV1/SERCA2a product. Special attention was placed on the inactivation and removal of adenovirus and its free components, clearance of DNA and protein impurities, and even intact host cells.

MYDICAR drug product is produced by an FDA registered contract manufacturer. The manufacturing process is relatively simple: drug product is diluted to a specified concentration, filter-sterilized, and vials are aseptically filled into single-use standard pharmaceutical grade vials and stoppered using an automated filling machine. The final drug product is stored frozen or refrigerated until use.

Our Plans for Scale-Up and Our Approach to Commercial Manufacturing

Our production process was successfully scaled up from lab scale to the 250-liter clinical scale. Of the limited number of batches produced at 250 liters, two batches were successfully produced at Targeted Genetics Corporation (now AmpliPhi Biosciences Corporation) in Seattle, Washington. We have transferred the manufacturing process to a contract manufacturer, Lonza Houston Inc., or LHI, in Houston, Texas. In December 2014, these activities culminated in the cGMP manufacture of a 2,000 liter batch with the primary objective of demonstrating scale-up and informing commercial facility design. Sample testing from the demonstration batch is currently in process. Two additional 2,000 liter batches are planned for production at LHI in 2015.

We are working with Lonza as our contract manufacturing organization for the production of AAV1/SERCA2a for our clinical studies. In addition, we have recently entered into an agreement with Novasep and are currently conducting the initial analytical and process transfer activities necessary for Novasep to achieve production of AAV1/SERCA2a.

The AAV1/SERCA2a manufacturing process is designed and operated using standard off-the-shelf equipment, including a 2,000-liter disposable bioreactor platform. The concept is to have a production train that can be replicated in standardized fashion to ensure that from facility to facility the manufacturing process is operated exactly the same using identical equipment, material and supplies. We anticipate that one production train will meet our global product requirements for our expected first indication, HFrEF. However, if actual product demand is greater than anticipated or additional indications gain approval, we believe that the standardized approach will allow for an easy and quick start-up of additional production trains. Our approach is designed to minimize capital costs and provide nimbleness and expandability of the production process.

MYDICAR Clinical and Commercial Supply

We currently have enough MYDICAR clinical supplies (drug product) to complete the MYDICAR-LVAD and AGENT-HF trials. We also have sufficient remaining drug product to provide clinical supplies for all of our currently planned Phase 1 and 2 clinical trials, including the high dose trial, the AAV NAb positive trial, the viral shedding trial, and the MYDICAR-AVF maturation trial, if commenced. At least one additional batch of drug substance and drug product will be required to conduct the CELL-003 trial and an HFpEF trial, if commenced.

Our current plan for commercial manufacturing is to potentially maintain dual source commercial supply relationships with Lonza, Novasep and/or other contract manufacturing organizations for long-term commercial supply. We have entered into agreements with Lonza and Novasep which provide for the commercial supply of AAV1/SERCA2a under various scenarios. The activities ultimately undertaken pursuant to these agreements will depend on our decision as to whether to proceed under these agreements. Our decision as to whether to proceed under one or both of these agreements will be impacted by a variety of factors, including the outcome of the

CUPID 2 trial, our expectations regarding clinical trial requirements and development timelines, the perception of the prospects for commercialization of MYDICAR and our ability to obtain financing and fund our financial obligations under the agreements.

Companion Diagnostic

The presence of pre-existing NAbs against the proteins that encapsulate the AAV1 gene therapy agent can block entry of the gene therapy agents into their target cells. Preclinical and limited clinical results with AAV1 NAb positive animals or patients, as well as *in vitro* neutralization experiments, have demonstrated that the detection of AAV1 NAbs is important prior to treatment with MYDICAR. Our experience in our CUPID 1 and CUPID 2 trials indicates that approximately 40% of the heart failure patients in the United States are AAV1 NAb negative and hence eligible for MYDICAR therapy. In other countries, such as Poland, the prevalence of pre-existing AAV1 NAbs is significantly higher.

We have developed a companion diagnostic AAV1 NAb assay for use in combination with MYDICAR in order to qualify subjects for treatment in clinical trials and for commercial use. The AAV1 NAb assay is intended to measure the loss of infectivity of AAV1/GFP (green fluorescent protein), an AAV1 recombinant particle with a reporter gene, following treatment with subject's serum (i.e., neutralization). Diluted samples of a subject's serum are incubated with AAV1/GFP, and then the mixture is tested for vector activity/infectivity *in vitro* on a permissive cell line (testing the relative gene expression (fluorescence) as a measure of vector neutralization).

To date, our tests to measure a potential clinical trial participant's level of pre-existing NAbs have been performed for us by Laboratory Corporation of America Holdings. We expect that the commercial assay, if approved, would be automated and similarly run by a strategic partner in at least two locations worldwide. It is not expected that the assay will be provided to the laboratories as a stand-alone kit but that approved laboratories would purchase the cells, controls and critical reagent, AAV1/GFP, from qualified suppliers. We intend that Quality System regulation set forth in 21 CFR Part 820 would be followed for the manufacture of AAV1/GFP and for the performance of the assay.

Companion diagnostics are subject to regulation by the FDA, the EMA and other foreign regulatory authorities as medical devices and require separate regulatory clearance or approval prior to commercial use. We anticipate that our companion diagnostic will require approval under a pre-market approval application, or PMA, submitted to the FDA's Center for Devices and Radiological Health, or CDRH, prior to commercialization. We further anticipate that regulatory approval of our companion diagnostic will be a prerequisite to our ability to market MYDICAR. Representatives of CDRH have participated in our meetings with the Center for Biologics Evaluation and Research, or CBER, regarding MYDICAR to discuss the potential use of our companion diagnostic, and we anticipate that future meetings will include representatives from both CBER and CDRH to ensure that the BLA submission (for MYDICAR) and PMA submission (for the companion diagnostic) are coordinated and subject to parallel review by these respective FDA centers. Accordingly, our objective is to align the development programs such that the companion diagnostic will be developed and approved contemporaneously with MYDICAR. In 2014, CE Marking was obtained for the companion diagnostic in Europe.

MYDICAR Administration Devices

MYDICAR is administered in an outpatient cardiac catheterization laboratory by a qualified cardiologist as a one-time, single dose, intracoronary infusion using a legally marketed syringe pump and off-the-shelf components typically used for minimally invasive interventional procedures, including a 60 mL syringe, tubing, stopcocks and appropriate percutaneous catheter. We are in discussions with the FDA regarding testing, packaging, and labeling requirements for the administration devices at the time of MYDICAR commercialization.

Competition

The biotechnology and pharmaceutical industries in which we operate are subject to rapid change and are characterized by intense competition to develop new technologies and proprietary products. We face potential competition from many different sources, including larger and better-funded pharmaceutical companies. While we believe that MYDICAR's unique mechanism of action provides us with competitive advantages, particularly given that MYDICAR is designed to be administered in conjunction with other pharmacological agents and devices (except LVADs), we have identified several companies which are active in the advancement of gene therapy products in the heart failure arena as of the date of this report. Not only must we compete with other companies that are focused on gene therapy treatments, any products that we may commercialize will have to compete with existing therapies and new therapies that may become available in the future.

Some of the pharmaceutical and biotechnology companies that are developing gene therapy products for the treatment of heart failure include Renova Therapeutics, NanoCor Therapeutics, Juventas Therapeutics, VentriNova and Beat BioTherapeutics. Renova, Beat BioTherapeutics and Juventas are in the clinical stages of development with their gene therapy products targeting moderate to advanced heart failure. Renova is using adenovirus serotype 5 encoding human adenylyl cyclase type 6 in a Phase 1/2 trial, while Juventas is enrolling a Phase 2 trial with its product candidate JVS100, which is a non-viral plasmid that encodes for stromal cell-derived factor-1 (SDF-1). UniQure (AAV delivery of S100A1), NanoCor (BNP delivery of I1), VentriNova (cyclin A2), and Beat BioTherapeutics (AAV/R1R2) are in the preclinical testing of product candidates. These companies also compete with us in recruiting human capital and securing licenses to complementary technologies that may be critical to the success of our business. They also compete with us for potential funding from the biotechnology and pharmaceutical industries. Our potential competitors also include academic institutions, government agencies and research institutions. In addition, as the presence of pre-existing NAbs against the proteins that encapsulate the AAV1 gene therapy agent can block entry of the AAV1 gene therapy agents into their target cells, previous patient exposure to other AAV1-based gene therapies, irrespective of the condition or disease they aim to treat, would render a patient ineligible for MYDICAR therapy and could therefore be considered competitive to MYDICAR.

We believe that the key competitive factors that will affect the development and commercial success of MYDICAR and any other product candidates that we develop are efficacy, safety and tolerability profile, convenience in dosing, product labeling, value, price and the availability of reimbursement from the government and other third-parties. Our commercial opportunity could be reduced or eliminated if our competitors have products which are better in one or more of these categories.

Intellectual Property

We strive to protect and enhance the proprietary technologies that we believe are important to our business, and seek to obtain and maintain patents for any patentable aspects of our products or product candidates, including our companion diagnostic, their methods of use and any other inventions that are important to the development of our business. Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, maintain our licenses to use intellectual property owned by third parties, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and other proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen, and maintain our proprietary position in the fields targeted by our product candidates.

We are the owner or licensee of a portfolio of patents and patent applications and possess substantial know-how and trade secrets which protect various aspects of our business. The patent families comprising our patent portfolio are primarily focused on MYDICAR for the treatment of heart failure and are generally directed to certain genes, AAV vectors and methods of delivering such AAV vectors to cells, methods of delivery to

myocardial cells and processes to manufacture our product candidates. We intend to leverage the intellectual property surrounding MYDICAR, together with the 12 years of available regulatory exclusivity that we expect to receive under the Biologics Price Competition and Innovation Act, as an important component of our business strategy.

Patent Protection for MYDICAR

Our portfolio of patents and patent applications related to MYDICAR generally relates to three aspects of MYDICAR: use of the SERCA2a gene for the treatment of heart failure; use and delivery of AAV vectors as a therapy; and manufacture of AAV vectors. The patent families which we believe are important for the protection of MYDICAR after its expected approval are summarized below. See also "Business—License Agreements."

- *Delivery of AAV Vectors to the Heart as a Therapy*. We are the sole owner of two patent families related to a method of treating cardiovascular disease by infusion of a therapeutic nucleic acid, such as MYDICAR, into the coronary circulation over a specified period of time, either alone or optionally with a vasodilating substance such as nitroglycerine. Two patents have issued from these families (U.S. Patent Nos. 8,221,738 and 8,636,998), which includes claims to the use of a vasodilator in conjunction with MYDICAR. These patents are expected to expire in July 2030 and October 2028, respectively. We are currently prosecuting other method of use applications, and we expect that an additional patent or patents will issue from these families. If issued, these patents would expire between 2027 and 2028, excluding any potential additional term that may be available as a result of patent term adjustments, or if we elect to seek patent term extensions, or PTEs, that may be available under the Hatch-Waxman Act. In addition to the United States, corresponding patents have issued in Europe (EP 2044199), Israel (IL 196541) and Japan (JP 5623740), and applications are pending in Australia, Europe, Hong Kong, India, and Japan. These patents and any patents issuing from the pending applications are expected to expire in July 2027 or October 2028.
- *Composition of MYDICAR*. MYDICAR utilizes a hybrid AAV vector, where the various components of the AAV vectors (capsid proteins and/or genetic material) are from different AAV serotypes. We in-licensed two patent families containing patent applications related to recombinant hybrid AAV vectors, the first via a sublicense from the University of Pennsylvania, or UPenn, under our exclusive license agreement with AmpliPhi (formerly Targeted Genetics), and the second under our non-exclusive license agreement with AskBio LLC, or AskBio. We expect that these patent families (U.S. Patent Nos. 6,759,237, 7,186,552 and 7,172,893) will expire in November 2019 and February 2021, and we expect to pay a royalty to UPenn and AskBio upon commercialization of MYDICAR. Foreign patents corresponding to U.S. Patent Nos. 6,759,237 and 7,186,522 have issued in Australia (AU 768729 and AU 2004201463), Canada (CA 2,349,838), Europe (EP 1127150) and Japan (JP 2000/58122700), all of which are expected to expire in November 2019. Foreign patents corresponding to U.S. Patent No. 7,172,893 include issued patents in Australia (AU 780231), Canada (CA 2348382) and Europe (EP 1135468), all of which are expected to expire in November 2019. In addition, U.S. Patent No. 8,637,255, a family member of the patents sublicensed from UPenn, has issued. This patent is directed to methods of assaying for the presence of NAbs specific against a recombinant AAV virion. This patent is expected to expire in December 2019.
- *Manufacture of AAV Vectors.* The manufacture and purification of the AAV vector used in MYDICAR is complicated and requires technical know-how. Our manufacturing process technology is protected by patents, trade secrets and proprietary know-how. We have obtained an exclusive license from AmpliPhi for certain aspects of the AAV manufacturing technology related to MYDICAR. This includes licenses to several patent families covering products and methods of manufacturing AAV vectors, including patent families related to stably transfected host cells for production of AAV vectors, and methods for commercial scale manufacturing and purification of recombinant AAV vectors. Taken in conjunction with our proprietary know-how, these patents are expected to offer additional protection by restricting competitors' access to AAV manufacturing methods used to make MYDICAR or

competing AAV-based products. In the United States, these patents (U.S. Patent Nos. 6,566,118, 6,989,264, 6,995,006 and 6,475,769) are expected to expire in September 2018. Corresponding foreign patents have issued in Australia (AU 758708, AU 772921, AU 2003204921), Canada (CA 2302992, CA 2342849), Europe (EP 1009808, EP 1109892), and Japan (JP 4472182), all of which are expected to expire in September of 2018 or 2019. Our exclusive license with AmpliPhi includes a patent family related to improved methods for purification of recombinant AAV vectors (WO 2010/148143), which, based on information provided by the licensor, we believe is pending in Brazil, Canada, China, Europe, Hong Kong, Israel, India, Japan, South Korea, Mexico, Russia, Singapore and the United States, and any resulting patents are expected to expire in June of 2030.

- *Use of SERCA2a for the Treatment of Heart Failure.* We are developing MYDICAR for the treatment or prevention of heart failure through the use of AAV vectors to deliver the SERCA2a gene to improve cardiac function. We have licensed certain patent rights from The Regents of the University of California (including U.S. Patent No. 7,745,416) related to gene therapy for the purpose of increasing SERCA2a expression in the treatment of heart failure, which have been important in the development of MYDICAR, but these patent rights are expected to expire in the United States in 2015 prior to our anticipated approval of MYDICAR. Corresponding patents have issued in Australia (AU 2004204815) and Israel (IL169663), with expected expiration in January 2024, and Canada (CA 2217967) and Europe (EP 0820310, EP 1977767) which are expected to expire in April 2016. An application is pending in Canada.

International Patent Protection for MYDICAR

We are the owner or licensee of numerous patents and patent applications in jurisdictions outside the United States. As noted above, most of the patent families discussed above have issued or are pending in foreign jurisdictions. Depending on the applicable national laws, these patents and patent applications (if applicable) covering MYDICAR may also benefit from extensions of patent term in individual countries.

Trade Secret Protection for MYDICAR

We exclusively in-license certain trade secret technology and know-how for manufacturing the AAV vector used in MYDICAR under our 2012 agreement with AmpliPhi. We believe that the expertise and materials licensed to us provide us with a commercial advantage over competitors attempting to utilize an AAV vector in their products.

U.S. Regulatory Protection for MYDICAR

In addition to patent and trade secret protection, we expect to receive a 12-year period of regulatory exclusivity from the FDA upon approval of MYDICAR pursuant to the Biologics Price Competition and Innovation Act. The exclusivity period, if granted, will run from the time of FDA approval. This exclusivity period, if granted, will supplement the intellectual property protection discussed above, providing an additional barrier to entry of any competitor seeking approval for a biosimilar version of MYDICAR.

In addition, it is possible to extend the patent term of one patent covering MYDICAR following FDA approval. This PTE is intended to compensate a patent owner for the loss of patent term during the FDA approval process. If eligible, we may use a PTE to extend the term of one of the patents discussed above beyond the expected expiration date, providing additional protection for MYDICAR.

Patent Protection of Pipeline Products

While the majority of our patent portfolio is related to MYDICAR and its use for treating heart failure, we are the owner or licensee of several additional patent families which relate to other technology which we are

developing, including our small molecule program and our stem cell factor program. This includes treatments for additional indications using SERCA enzymes and MYDICAR, and new drugs for treating other SERCA-related diseases.

- *Methods of Treating Stenosis*. We in-license a patent family from The General Hospital Corporation related to using SERCA2a genes, including delivery by AAV vectors, to reduce stenosis, which is the narrowing of a blood vessel, or restenosis, which is the repeated narrowing in blood vessels. We expect that these patents (U.S. Patent Nos. 7,291,604 and 8,133,878) will expire no earlier than September 2024.
- *Methods of Treating Pulmonary Arterial Hypertension*. We are the co-owner with the Mount Sinai School of Medicine of New York University, or Mount Sinai, of a patent family containing patent applications (U.S. Patent Pub. 2011/0256101) related to the use of genes, including SERCA, to treat pulmonary arterial hypertension, a type of high blood pressure that affects the arteries in the lungs and the right side of the heart. These applications are currently in prosecution, and we expect that any patents that may issue from this family of patent applications will expire no earlier than April 2031. We are the exclusive licensee of Mount Sinai's joint ownership interest in this patent family pursuant to a license agreement.
- *Methods of Treating Heart Arrhythmia*. We in-license a patent family from The General Hospital Corporation containing patent applications (U.S. Patent Pub. 2009/0239940) which disclose methods and materials for treating heart disease, including heart arrhythmia, using SERCA2a and AAV vectors. The case was unintentionally abandoned and is in the process of being revived through the U.S. Patent and Trademark Office. We expect that any patents which issue from this family of patent applications will expire no earlier than July 2018.
- *Activation of SERCA2a using Zinc Finger Technology*. We are the sole owner of a patent family containing a patent application (U.S. Patent Pub. 2011/0172144) related to the use of a class of proteins known as zinc finger proteins to augment the expression of SERCA2a in cardiac muscle. Filed in January of 2011, we expect that any patent which issues from this application will expire no earlier than January of 2031.
- *High-throughput Screening for SERCA Modulators and Their Use*. We are the co-owner, with The Regents of the University of Minnesota, or UMinn, of a patent family (U.S. Patent No. 8,431,356) that relates to high-throughput screening methods used to identify small molecule compounds that modulate SERCA activity, as well as their use in treating SERCA-related disease. We are the exclusive licensee of UMinn's joint ownership interest in these patents pursuant to a license agreement and we are solely responsible for the prosecution of these patents. We plan to use this technology to help identify product candidates which can be used to increase SERCA activity in muscle tissue, including the heart, to build a pipeline of SERCA-related therapies. We expect patents that may issue from these patent families to expire no earlier than January 2030. The current issued patent will expire in January 2030.
- *Methods of Treating Ischemic Diseases Using Stem Cell Factor Coding Sequences.* We have been assigned certain patent rights from Enterprise related to certain gene therapy applications of the membrane-bound form of the Stem Cell Factor gene for treatment of cardiac ischemia. Included within these rights is U.S. Patent No. 8,404,653, which has a projected expiration date of April 2029. Similar applications are pending in Europe (Publication No. EP1948246A2) and Hong Kong (Application No. 09100868.0).

Trademarks

We have registered the trademark "MYDICAR" in the United States for use in connection with a biological product, namely, a gene transfer product composed of a recombinant AAV vector for medical use. We intend to pursue additional registrations in markets outside the United States where we plan to sell MYDICAR.

Patent Term

The term of individual patents and patent applications listed in previous sections will depend upon the legal term of the patents in the countries in which they are obtained. In most countries, the patent term is 20 years from the date of filing of the patent application (or parent application, if applicable). For example, if an international Patent Cooperation Treaty, or PCT, application is filed, any patent issuing from the PCT application in a specific country expires 20 years from the filing date of the PCT application. In the United States, however, if a patent was in force on June 8, 1995, or issued on an application that was filed before June 8, 1995, that patent will have a term that is the greater of 20 years from the filing date, or 17 years from the date of issue.

Under the Hatch-Waxman Act, the term of a patent that covers an FDA-approved drug or biological product may also be eligible for PTE. PTE permits restoration of a portion of the patent term of a U.S. patent as compensation for the patent term lost during product development and the FDA regulatory review process if approval of the application for the product is the first permitted commercial marketing of a drug or biological product containing the active ingredient. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. The Hatch-Waxman Act permits a PTE for only one patent applicable to an approved drug, and the maximum period of restoration is five years beyond the expiration of the patent. A PTE cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, and a patent can only be extended once, and thus, even if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions may be available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug. When possible, depending upon the length of clinical trials and other factors involved in the filing of a BLA, we expect to apply for PTEs for patents covering our product candidates and their methods of use.

For additional information on PTE, see “Business—Government Regulation.”

Proprietary Rights and Processes

We may rely, in some circumstances, on proprietary technology and processes (including trade secrets) to protect our technology. However, these can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with those who have access to our confidential information, including our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our proprietary technology and processes by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our proprietary technology and processes may otherwise become known or be independently discovered by competitors. To the extent that our employees, consultants, scientific advisors, contractors, or any future collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions. For this and more comprehensive risks related to our proprietary technology and processes, please see “Risk Factors—Risks Related to Our Intellectual Property”.

License Agreements

License Agreement with The Regents of the University of California

In February 2001, we entered into a license agreement with The Regents of the University of California, or UC, under which we obtained an exclusive, worldwide license to UC’s patent rights in certain inventions, or the UC Patent Rights, related to the use of gene therapy vectors to deliver the SERCA2a gene to improve cardiac function, including certain patents related to MYDICAR. The agreement was amended twice, once in March 2001 to modify certain financial terms and once in January 2005 to make further amendments to the financial terms, with the second amendment also adding additional patents. We paid to UC an amendment fee of $114,455

and reimbursed UC for approximately $86,000 of previously incurred patent costs relating to the UC Patent Rights in connection with the second amendment of the agreement in January 2005.

Under the agreement, we are permitted to research, develop, manufacture and commercialize products utilizing the UC Patent Rights for gene therapy for the treatment or prevention of heart failure by the delivery of a gene or a synthetic equivalent, including SERCA2a, and to sublicense such rights. UC retained the right, on behalf of itself and other non-profit institutions, to use the UC Patent Rights for educational and research purposes and to publish information about the inventions covered by the UC Patent Rights.

In consideration for the rights granted to us under the agreement, we issued an aggregate of 83 shares of our common stock to UC upon the achievement of certain developmental milestones. We are required to issue to UC an additional 55 shares of our common stock and pay to UC up to an aggregate of approximately $1.6 million upon the achievement of certain developmental and regulatory milestones. In addition, upon commercialization of any product utilizing the UC Patent Rights, we will be required to pay to UC a low single-digit royalty on net sales of such product sold by us or our affiliates subject to minimum annual royalty payments and other adjustments in certain circumstances. However, we do not expect to commercialize MYDICAR prior to the expiration of the UC Patent Rights applicable to MYDICAR in the United States and Europe. Our obligation to pay milestones and royalties to UC terminates upon the expiration of the applicable UC Patent Rights.

In the event we sublicense a UC Patent Right, we are obligated to pay to UC a fee based on a percentage of sublicense fees received by us, which percentage ranges from the low-teens to mid-twenties depending on the country of origin of such UC Patent Right and is subject to adjustment in certain circumstances. In addition, we will also be required to pay to UC a low single-digit percentage sublicense royalty on net sales of products sold by our sublicensees that utilize the sublicensed UC Patent Right, but in no event will we be required to pay more than 50% of the royalties we receive from such sublicensees.

The agreement requires that we diligently develop, manufacture and commercialize products that are covered by the UC Patent Rights, and we have agreed to meet certain developmental and commercial milestones. UC may, at its option, either terminate the agreement or change the license granted from an exclusive license to a non-exclusive license if we fail to meet such milestones. We are currently in compliance with these milestone requirements.

We may unilaterally terminate the agreement for any reason upon 90 days' written notice to UC. UC may terminate the agreement in the event of our nonperformance or breach of the agreement if such nonperformance or breach remains uncured for 60 days following our receipt of written notice of such nonperformance or breach. Absent early termination, the agreement will continue until the expiration date of the longest-lived patent right included in the UC Patent Rights, which is expected to occur in 2024.

Exclusive License Agreement with Dr. Martin J. Kaplitt

In June 2006, we entered into an exclusive license agreement with Dr. Martin J. Kaplitt pursuant to which Dr. Kaplitt granted to us an exclusive, worldwide license under Dr. Kaplitt's interest in certain patents related to the use of AAV vectors to deliver genes to cardiac muscles and delivery methods of AAV vectors to heart cells for the development, manufacture, use and sale of MYDICAR. The license granted to us under the agreement automatically became non-exclusive on the fourth anniversary of the effective date of the agreement. We have the right to grant sublicenses to third parties under the agreement.

In consideration for the rights granted to us under the agreement, we paid an upfront fee to Dr. Kaplitt of $25,000. We are also obligated to pay to Dr. Kaplitt an annual license maintenance fee of $6,000 during the term of the agreement. In addition, we are required to pay to Dr. Kaplitt a very low single-digit percentage royalty on net sales of products sold by us, our affiliates and our sublicensees that are covered by the licensed patents. Our royalty obligations continue on a product-by-product and country-by-country basis until the expiration of the

last-to-expire valid claim in the licensed patents covering a licensed product in such country. Finally, we are obligated to pay to Dr. Kaplitt up to an aggregate of $200,000 upon the achievement of certain regulatory milestones.

We may unilaterally terminate the agreement upon 60 days' written notice to Dr. Kaplitt. Dr. Kaplitt may terminate the agreement in the event of our material breach of the agreement if such breach remains uncured for 60 days following our receipt of written notice of such breach. Absent early termination, the agreement will automatically terminate upon the expiration of the last-to-expire of the licensed patents containing a valid claim, which is expected to occur in 2015, prior to the projected launch of our product candidates.

Sublicense Agreement and Amended and Restated License Agreement with AmpliPhi

Sublicense Agreement

In June 2012, we entered into a sublicense agreement with AmpliPhi, or the AmpliPhi Sublicense, pursuant to which AmpliPhi sublicensed to us certain rights under a separate agreement, the UPenn Agreement, which AmpliPhi entered into in 2009 with the Trustees of UPenn. Under the terms of the agreement, we obtained an exclusive, worldwide sublicense from AmpliPhi under certain UPenn patents related to AAV1 vectors for the development, manufacture, use and sale of companion diagnostics to MYDICAR. We have the right to grant sublicenses to our affiliates and third-party collaborators under the agreement solely for research, development or other non-commercial purposes, or as reasonably necessary, to our manufacturers or distributors, provided that we remain primarily liable and such downstream sublicenses are consistent with the terms of our agreement with AmpliPhi and prohibit further sublicensing. In addition, we are required to use commercially reasonable efforts to meet certain developmental, regulatory and commercial milestones with respect to companion diagnostics under the agreement. We are currently in compliance with these milestone requirements. While we have sole control over the development and commercialization of companion diagnostics under the agreement, AmpliPhi has the first right to prosecute and maintain the licensed patents, subject to our right to consult with AmpliPhi with regard to such prosecution and maintenance upon our reasonable request.

In consideration for the sublicense granted to us under the agreement, we paid to AmpliPhi a sublicense initiation fee of $310,000, and we are obligated to pay to AmpliPhi an annual sublicense maintenance fee of $310,000. We are also required to pay to AmpliPhi a low single-digit percentage royalty based on net sales of any companion diagnostic covered by a licensed patent sold by us, our affiliates or our sublicensees. Our royalty obligations continue on a companion diagnostic-by-companion diagnostic and country-by-country basis until the expiration of the last-to-expire valid claim in a licensed patent covering the applicable companion diagnostic in such country. Finally, we are obligated to pay to AmpliPhi all royalty and milestone payments that become due and payable by AmpliPhi to UPenn under the UPenn Agreement as a result of our exercise of the sublicense granted under our agreement with AmpliPhi, including a low single-digit tiered percentage royalty on net sales of any companion diagnostic sold by us, our affiliates or our sublicensees, which royalty is separate from and in addition to the royalty payable to AmpliPhi described above, and up to an aggregate of $850,000 in potential milestone payments per product covered by the licensed patents.

We may unilaterally terminate the agreement upon 30 days' written notice to AmpliPhi. Absent early termination, the agreement will automatically terminate upon the expiration of the last-to-expire licensed patent, which is expected to occur in 2019.

Amended and Restated License Agreement

We entered into an amended and restated license agreement with AmpliPhi concurrently with the AmpliPhi Sublicense that both amended the terms of the license agreement which we entered into with AmpliPhi in 2009 and terminated our manufacturing agreement with AmpliPhi which we entered into in 2009. Under the agreement, we obtained an exclusive, worldwide license under certain patents and know-how related to

AmpliPhi's AAV vector and manufacturing technology for the development, manufacture, use and sale of MYDICAR. We have the right to grant sublicenses to our affiliates and third-party collaborators under the agreement for research, development or other non-commercial purposes, or as reasonably necessary, to our manufacturers or distributors, provided that we remain primarily liable and such sublicenses comply with the terms of our agreement with AmpliPhi and prohibit further sublicensing. In addition, we have agreed to use commercially reasonable efforts to meet certain diligence milestones with respect to the development and commercialization of at least one product covered by the UPenn patent rights licensed to AmpliPhi by UPenn under the UPenn Agreement. We are currently in compliance with these milestone requirements. While we have sole control over development and commercialization of products covered by the licensed patents, AmpliPhi has the first right to prosecute and maintain the licensed patents, subject to our right to consult with AmpliPhi with regard to such prosecution and maintenance upon our reasonable request.

During the term of the agreement, we are obligated to pay to AmpliPhi all royalty and milestone payments that become due and payable by AmpliPhi to UPenn under the UPenn Agreement as a result of our exercise of the sublicense granted under our agreement with AmpliPhi. This includes a low single-digit tiered percentage royalty on net sales of MYDICAR and any other product covered by the licensed patents sold by us, our affiliates or our sublicensees, and up to $850,000 in milestone payments upon the achievement of certain developmental and regulatory milestones related to MYDICAR and any other product covered by the licensed patents.

The agreement does not provide either party with termination rights and does not have a provision for expiration or automatic termination.

License Agreement with AdVec

In February 2009, we entered into a license agreement with AdVec, Inc., or AdVec, under which we obtained a non-exclusive, worldwide license to use and acquire from AdVec's distributor certain human embryo kidney cells transformed by Adenovirus 5 DNA, or 293 Cells, and certain AdVec know-how related to 293 Cells for use in testing of MYDICAR for lot release. In consideration for the rights granted to us under the agreement, we are obligated to pay to AdVec an annual license maintenance fee of $5,000.

Either party may terminate the agreement upon written notice of the other party's insolvency or bankruptcy or upon the other party's breach of the agreement if such breach remains uncured after 60 days of receipt of written notice of such breach. Absent early termination, the agreement will remain in effect until the tenth anniversary of the effective date. Thereafter, the agreement will automatically renew for successive five-year terms unless either party notifies the other party in writing at least 90 days prior to the end of any such five-year term of its election not to renew the agreement.

Non-Exclusive License Agreement with Virovek

In November 2010, we entered into a non-exclusive license agreement with Virovek Incorporation, or Virovek, under which we obtained a non-exclusive, worldwide license under certain patent rights and trade secrets related to Virovek's AAV baculovirus technology to develop, manufacture, use and sell AAV1/GFP vector reagents as part of a companion diagnostic. Under the terms of the agreement, we have the right to grant sublicenses to third parties, and we are required to use commercially reasonable efforts to develop and commercialize a companion diagnostic to MYDICAR. We are currently in compliance with this requirement.

In consideration for the rights granted to us under the agreement, we paid to Virovek an up-front license fee of $15,000, and we are obligated to pay to Virovek an annual maintenance fee of $20,000, which fee is creditable against royalties due under the agreement. We are also required to pay to Virovek a percentage royalty in the mid-teen range based on upfront, annual, milestone, royalty and other payments received by us as a result of the performance of companion diagnostics by us, our affiliates and our sublicensees, subject to adjustment in certain circumstances. Our royalty obligations continue on a companion diagnostic-by-companion diagnostic and

country-by-country basis until the expiration of the last-to-expire valid claim in a licensed patent covering the companion diagnostic in such country, which is expected to occur in 2027, or 10 years from the date of first commercial sale in such country if the companion diagnostic is covered only by licensed trade secrets.

We may unilaterally terminate the agreement upon 60 days' notice to Virovek. Either party may terminate the agreement for the other party's material breach of the agreement if such breach remains uncured after 90 days of receiving written notice of such breach. Absent early termination, the agreement will automatically terminate upon the expiration of our royalty payment obligations.

Non-Exclusive License Agreement with AskBio

In January 2008, we entered into a non-exclusive license agreement with AskBio, a wholly owned subsidiary of Asklepios Biopharmaceutical Inc., under which we obtained a non-exclusive, worldwide license under certain patents related to recombinant AAV vectors to develop, manufacture, use and sell MYDICAR. We have the right to grant sublicenses to third parties under the agreement provided that such sublicenses are entered into pursuant to a written sublicense agreement containing terms consistent with our agreement with AskBio.

Under the terms of the agreement, we granted to AskBio an option to obtain a non-exclusive, worldwide license under certain of our patent rights related to infusion of AAV in the arteries of the heart to develop, manufacture, use and sell products for the treatment of cardiac diseases. This option includes our currently pending patent application related to a method of treating cardiovascular disease by infusion of a therapeutic nucleic acid into the coronary circulation over a specified period of time. It does not include our issued patent in this family, which includes claims to the concurrent use of a vasodilating substance such as nitroglycerine. If AskBio timely exercises its option to obtain the license under the agreement on or before the earlier of January 15, 2015 and within 60 days following notice that a patent has issued from the patent applications included within the patent rights subject to the option, we will enter into a separate license agreement with AskBio with respect to such license with previously agreed upon payment terms. Although the scope of the license granted to AskBio upon exercise of the option would enable AskBio to develop and commercialize a competing product with respect to the patent rights to which the option applies, we believe that the exclusion of our issued patent from that license, and the scope of our anticipated regulatory approvals, will prevent AskBio from being able to launch any product that is able to compete directly with MYDICAR.

In consideration for the rights granted to us under the agreement, we paid to AskBio license fee payments of $150,000 in the aggregate. In addition, we are obligated to pay to AskBio an annual maintenance fee of $100,000. Upon commercialization of any product utilizing the licensed patents, we will also be required to pay to AskBio a low single-digit percentage royalty on net sales of such products, including MYDICAR. Our royalty obligations continue on a product-by-product and country-by-country basis until the expiration of the last-to-expire valid claim in a licensed patent covering the applicable product in such country, which is expected to be in 2021. We are also obligated to reimburse AskBio for up to an aggregate of $355,000 per licensed product upon the achievement of certain clinical, regulatory and sales milestones that may become due and payable by AskBio under a separate agreement between AskBio and the University of North Carolina at Chapel Hill from 2003.

We may unilaterally terminate the agreement upon 180 days' written notice to AskBio. Either party may terminate the agreement for the other party's material breach of the agreement if such breach is not cured after 30 days of receiving written notice of such breach. Absent early termination, the agreement will continue in effect until the expiration of our royalty payment obligations under the agreement.

Exclusive Patent License with the Regents of the University of Minnesota

We are joint owners with UMinn of the rights in a certain patent related to screening technology for isolation of small molecule modulators of SERCA enzymes (fluorescence resonance energy transfer, or FRET, assays). In May 2009, we entered into an exclusive patent license agreement with UMinn under which we

obtained an exclusive license to UMinn's joint ownership interest in the patent application that led to the current issued patent. We have the right to grant sublicenses to third parties under the agreement, and UMinn retained the right to use the licensed technology for non-commercial research and educational purposes.

We have agreed to meet certain performance milestones under the agreement, the deadline for which may be extended at our request provided that we have used commercially reasonable efforts to achieve such milestones by the applicable deadlines. We are currently in compliance with these milestone requirements. We have the first right to prosecute and maintain the applicable patent family.

In consideration for the rights granted to us under the agreement, we made an upfront payment to UMinn of $120,000. In addition, we are obligated to pay to UMinn an annual license fee of $120,000. The annual license fee will increase to $325,000 if we (1) undergo a change of control, (2) assign the agreement, any of our rights or obligations under the agreement or our joint ownership interest in the licensed technology, (3) receive a certain amount in license and sublicense revenues under the agreement, (4) file an IND, new drug application, or NDA, BLA or orphan drug application (or a foreign equivalent of any such application) for a product covered by the licensed technology, or (5) enter into any agreement with a third party to market or use the licensed technology, subject to certain exceptions.

We may unilaterally terminate the agreement upon 90 days' written notice to UMinn. UMinn may terminate the agreement upon 10 days' written notice to us upon our insolvency or for our breach of the agreement if such breach remains uncured for 90 days after we receive notice of such breach, or 30 days in the case of a non-payment breach. Absent early termination, the agreement will automatically terminate upon the expiration of all active claims in any licensed patent or patent application, which is expected to occur no earlier than January 2030.

Material Transfer and Exclusivity Agreement with Les Laboratoires Servier

In February 2014, we and Servier entered into a material transfer and exclusivity agreement, pursuant to which we agreed to transfer to Servier samples of certain proprietary compounds from our small molecule SERCA2b modulator program and granted to Servier a non-exclusive, non-sublicensable, royalty-free license to conduct certain studies of the samples for the purpose of evaluating Servier's interest in negotiating a potential license and research collaboration agreement with us relating to small molecule SERCA2b modulators, or Compounds, for the treatment of type 2 diabetes and other metabolic diseases. Although the evaluation period under this Agreement has expired, we are in the process of completing certain pre-clinical studies of these compounds in coordination with Servier and Servier is continuing to evaluate its potential interest in this program.

Exclusive Patent License with Enterprise Partners

On July 18, 2014, we and Enterprise entered into an Assignment and License Agreement, pursuant to which Enterprise granted to us an exclusive, worldwide license and the assignment of patents held by Enterprise relating to certain gene therapy applications of mSCF for the treatment of cardiac ischemia. We have the right to grant sublicenses to third parties under the agreement.

In consideration for the rights granted to us under the agreement, we paid an upfront fee to Enterprise of $160,000. We are also obligated to pay to Enterprise a milestone payment in the amount of $1,000,000 upon the grant to us, or an affiliate or sublicensee of ours, of the first regulatory approval in the United States of a product that is covered by the licensed patents. In addition, we are required to pay to Enterprise a 2% royalty on net sales of products sold by us or by our affiliates or sublicensees that are covered by the licensed patents. Our royalty obligations continue on a product-by-product and country-by-country basis until the expiration of the last-to-expire valid claim in the licensed patents covering a licensed product in such country.

We may unilaterally terminate the agreement upon written notice to Enterprise. Enterprise may terminate the agreement in the event of our material breach of the agreement if such breach remains uncured for 90 days following receipt of written notice of such breach. Absent early termination, the agreement will automatically terminate upon the expiration of the last-to-expire of the licensed patents containing a valid claim.

Manufacturing

Manufacturing Services Agreement with Lonza

In August 2012, we entered into a manufacturing services agreement with Lonza, which we subsequently amended and restated in August 2013. Under the terms of the agreement, Lonza provides manufacturing services to produce MYDICAR at a scale sufficient for our clinical trials to date. We pay for manufacturing services performed by Lonza under the agreement pursuant to statements of work entered into from time to time.

We may unilaterally terminate the agreement upon six months' written notice to Lonza. Lonza may terminate the agreement upon written notice to us, provided that such termination by Lonza will not be effective until the earlier of one year after the date we receive such written notice or our qualification of an alternative supplier and completion of certain technology transfer assistance services to establish manufacturing capabilities at the alternative supplier's facilities. Either party may terminate the agreement in the event of the other party's insolvency or for the other party's material breach of the agreement if such breach remains uncured after 30 days of receiving written notice of such breach or after 180 days of receiving written notice of such breach if such breach is not a non-payment related breach, is not capable of being cured within 30 days and the breaching party is making diligent efforts to cure such breach. In addition, either party may terminate the agreement, by providing two months' written notice to the other party if it receives notice that the production of MYDICAR under the agreement or clinical trials for which MYDICAR is being produced has been or will be suspended or terminated by the FDA or EMA due to product failure. Absent early termination, the agreement will continue until the fifth anniversary of the effective date of the original agreement.

Facility Construction and Commercial Supply Agreement with Lonza

On October 31, 2014, we entered into a Facility Construction and Commercial Supply Agreement with Lonza, pursuant to which the parties agreed to initiate detailed design planning, or the Detailed Design, for the potential construction of a new commercial viral therapeutics facility in Portsmouth, New Hampshire for the manufacture of MYDICAR drug substance (AAV1/SERCA2a), and in exchange for an upfront reservation fee payable by us to Lonza, Lonza agreed to reserve, for a period of time extendable on payment of specified reservation extension fees, the capital, property and labor resources necessary to enable the initiation of construction of the facility within 75 days of receipt of notice of our decision to exercise the construction trigger and commit to a long-term supply arrangement for MYDICAR.

The construction trigger may not be exercised by us prior to completion of the Detailed Design for the facility, which is currently expected to be completed in the third quarter of 2015. If we exercise the construction trigger, Lonza would be obligated to purchase, subject to any applicable Nasdaq limitations, $10,000,000 worth of newly issued, unregistered shares of our common stock at a volume-weighted average market price and initiate construction of the facility. In exchange, we would be obligated to (i) fund Lonza's construction of the facility through time and event-triggered milestone payments secured by funds deposited us into an escrow account upon exercise us of the construction trigger, (ii) upon completion of the facility, fund Lonza's costs for overhead, including personnel reserved for manufacture of MYDICAR at the facility, and (iii) through such overhead funding arrangement, order from Lonza a certain percentage of our and our partners' annual global commercial supply of MYDICAR, subject to certain limits and adjustments.

The agreement would continue in effect until the earlier of the sixth anniversary of the first approval of MYDICAR in the United States or European Union, or the First Approval, or expiration of the reservation period

for construction of the facility prior to our exercising the construction trigger, subject to earlier termination under specified circumstances set forth in the agreement as described below. Additionally, if we exercise the construction trigger and are paying an agreed threshold for overhead for manufacture of MYDICAR at the facility, we have the right to extend the term of the agreement for an additional three years upon notice provided to Lonza between the third and fourth anniversary of the First Approval. We have the right to terminate the agreement (i) immediately upon notice to Lonza at any time prior to exercise of the construction trigger; (ii) upon 90 days' notice to Lonza if at any time we discontinue development and, if applicable, commercialization of MYDICAR as a result of regulatory, safety and/or efficacy concerns; or (iii) immediately upon notice to Lonza in the event of certain specified material breaches of the agreement by Lonza or Lonza's debarment. Additionally, each party may terminate the agreement upon uncured material breach of the agreement by, or upon the insolvency or bankruptcy of, the other party, or in the event of a continuing force majeure preventing performance. Upon any termination following exercise of the construction trigger other than for material breach of the agreement or Lonza's debarment, we are obligated to pay specified termination fees as set forth in the agreement.

Development, Manufacturing and Commercial Supply Agreement with Novasep

On March 20, 2015, we entered into a Development, Manufacturing and Supply Agreement with Novasep, which superseded our Letter Agreement with Novasep dated December 19, 2014. Under the terms of the Manufacturing Agreement, the parties agreed to continue the work initiated under the Letter Agreement, including the work necessary to prepare for the potential manufacture of MYDICAR drug substance (AAV1/SERCA2a) at the facilities of Novasep's affiliate Henogen in Europe (the "***Novasep Facility***"). Pursuant to the Manufacturing Agreement (and as previously agreed in the Letter Agreement), in exchange for payments from us to Novasep totaling up to €4,750,000, Novasep agreed to (i) conduct the engineering design work for facility modifications that would be necessary for the manufacture of MYDICAR drug substance, (ii) undertake initial process and analytical transfer and initial scale-up work in support of such potential future commercial manufacturing of MYDICAR drug substance, and (iii) allocate the resources and capacity necessary for the foregoing activities. The parties have also agreed to proceed with the additional process transfer, engineering/construction, scale-up and development activities necessary for future production of MYDICAR drug substance in accordance with current Good Manufacturing Practices ("***GMP***"), and agreed to terms of a commercial supply arrangement with a term through at least December 31, 2018, with extension options through 2020 in favor of us. We have the right to terminate the Manufacturing Agreement, exercisable for a specified period of time following the un-blinding of the data from our Phase 2b clinical trial of MYDICAR (CUPID 2), if we conclude in good faith that the CUPID 2 data is such that we do not require production of MYDICAR drug substance at the Novasep Facility.

Unless we exercise the post CUPID 2 data termination right described above, we will be obligated to (i) fund Novasep's modifications to the Novasep Facility through time-and event-triggered milestone payments, (ii) make additional payments for the development services to be performed by Novasep, and (iii) commit to purchase a specified number of batches of MYDICAR drug substance (or make minimum payments with respect to any such batches that are not purchased) through 2018 (if we elect that the Novasep Facility be operated as a multi-product facility) or through 2019 (if we elect to have the Novasep Facility dedicated to MYDICAR drug substance production during the term of the Manufacturing Agreement).

In addition to the above-described post CUPID 2 data termination right, we have the right to terminate the Manufacturing Agreement (i) at will on or before March 31, 2016, (ii) following the shut-down or non-production of the Novasep Facility for a specified period of time, or (iii) upon Novasep's debarment. Additionally, each party may terminate the Manufacturing Agreement upon uncured material breach thereof by the other party, upon the other party's insolvency or bankruptcy, or in the event of a continuing force majeure preventing performance. Upon any termination of the Manufacturing Agreement by us following the expiration of the post CUPID 2 data termination right either for convenience or for any reason other than material breach of the Manufacturing Agreement, shut-down or non-production of the Novasep Facility for a period extending

longer than six months, or Novasep's insolvency, we are obligated to pay previously-unreimbursed amounts incurred by Novasep and specified termination fees as set forth in the Manufacturing Agreement.

Government Regulation

Biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, and the Public Health Service Act, or PHS Act, and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, purity, potency, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biological products. FDA approval must be obtained before clinical testing of a biological product begins, and each clinical trial protocol for a gene therapy product is reviewed by the FDA and, in some instances, the U.S. National Institutes of Health, or NIH, through its Recombinant DNA Advisory Committee, or RAC. FDA approval also must be obtained before marketing of biological products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals. To date, the FDA has never approved a gene therapy product for commercial sale. Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products, CDRH regulates companion diagnostics, and the Office of Combination Products, or OCP, issues classification and jurisdiction assignments for medical products. Specifically, OCP determines how combination products, such as biologic/medical device combination products, will be regulated and which FDA Center or Lead Center (e.g., CBER or CDRH) will regulate the product.

CBER works closely with the NIH and its RAC, which makes recommendations to the NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. The FDA also has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing and chemistry, manufacturing and control information in gene therapy INDs.

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

U.S. Biological Products Development Process

The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to good laboratory practices, or GLPs, and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND application, which must become effective before human clinical trials may begin;

- performance of adequate and well-controlled human clinical trials according to the FDA's regulations, commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;
- submission to the FDA of a BLA for marketing approval that includes substantive evidence of safety, efficacy, purity and potency from results of nonclinical testing and clinical trials;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with GMP, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity;
- potential FDA audit of the nonclinical study and clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA.

Before testing any biological product candidate, including a gene therapy product, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs.

Where a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documentation is submitted to and the trial is registered with the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA, however many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH is responsible for convening the RAC, a federal advisory committee, which discusses protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify the FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA website and may be accessed by the public.

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. With gene therapy protocols, if the FDA allows the IND to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such trials.

Clinical trials involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse

events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. Clinical trials also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1.* The biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- *Phase 2*. The biological product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- *Phase 3*. Clinical trials are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire, of trial subjects.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or *in vitro* testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk, including risks inferred from other unrelated gene therapy trials. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biological product has been associated with unexpected serious harm to patients.

Human gene therapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the trial period, the number of patients the FDA will require to be enrolled in the trials in order to establish the safety, efficacy, purity and potency of human gene therapy products, or that the data generated in these trials will be acceptable to the FDA to support marketing approval. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System which includes information on gene transfer studies and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these studies.

Concurrently with clinical trials, companies usually complete additional animal studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with GMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

After the completion of clinical trials of a biological product, FDA approval of a BLA must be obtained before commercial marketing of the biological product. The BLA must include results of product development, laboratory and animal studies, human trials, information on the manufacture and composition of the product, proposed labeling and other relevant information. The FDA may grant deferrals for submission of data or full or partial waivers. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. The FDA adjusts the PDUFA user fees on an annual basis. PDUFA also imposes an annual product fee for biological products and an annual establishment fee on facilities used to manufacture prescription biological products. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with GMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS. The FDA will not approve a BLA without a REMS, if required.

Before approving a BLA, the FDA may inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with GMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements. To assure GMP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a risk management plan, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical trials, sometimes referred to as Phase 4 clinical trials, designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The FDA has agreed to certain review goals under PDUFA, and aims to complete its review of 90% of standard BLAs within 10 months from filing and 90% of priority BLAs within six months from filing. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests, or the BLA sponsor otherwise provides, additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Fast Track Designation, Accelerated Approval, Priority Review and Breakthrough Therapy Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug or biological product may request the FDA to designate the drug or biological product as a Fast Track product at any time during the clinical development of the product. Unique to a Fast Track product, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Other types of FDA programs intended to expedite development and review, such as priority review, accelerated approval and Breakthrough Therapy designation, also exist. A product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products.

The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical trials establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials to confirm the effect of the endpoint. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

A product may also be eligible for receipt of a Breakthrough Therapy designation. The Breakthrough Therapy designation is intended to expedite the FDA's review of a potential new drug for serious or life-threatening diseases or conditions where "preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development." The designation of a drug as a Breakthrough Therapy provides the same benefits as are available under the Fast Track program, as well as intensive FDA guidance on the product's development program. Fast Track designation, priority review, accelerated approval and Breakthrough Therapy designation do not change the standards for approval, but may expedite the development or approval process.

Combination Products

MYDICAR and certain delivery device components may be regulated as combination products. Combination products include products where two or more separate products are packaged together (e.g., drug and device products); or a product packaged separately but intended for use only with an approved, individually specified product, where both are required to achieve the intended use, indication, or effect and where upon approval of the proposed product, the labeling of the individually specified product would need to be changed (e.g., to reflect a change in intended use).

Regulation of Companion Diagnostics

In the United States, the FD&C Act and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import, and post-market surveillance. Companion diagnostic tests are classified as medical devices under the FD&C Act. Unless an exemption applies, diagnostic tests require marketing clearance or approval from the FDA prior to commercial distribution. The two primary types of FDA marketing authorization applicable to a medical device are premarket notification, also called 510(k) clearance, and PMA approval. We anticipate that the companion diagnostic tests we are developing will be subject to the PMA approval process.

PMA applications must be supported by valid scientific evidence, which typically requires extensive data, including technical, preclinical, clinical and manufacturing data, to demonstrate to the FDA's satisfaction the safety and effectiveness of the device. For diagnostic tests, a PMA application typically includes data regarding analytical and clinical validation studies. As part of its review of the PMA, the FDA will conduct a pre-approval inspection of the manufacturing facility or facilities to ensure compliance with the Quality System Regulation, or QSR, which requires manufacturers to follow design, testing, control, documentation and other quality assurance procedures. FDA review of an initial PMA application is required by statute to take between six to ten months,

although the process typically takes longer, and may require several years to complete. If the FDA evaluations of both the PMA application and the manufacturing facilities are favorable, the FDA will either issue an approval letter or an approvable letter, which usually contains a number of conditions that must be met in order to secure the final approval of the PMA. If the FDA's evaluation of the PMA or manufacturing facilities is not favorable, the FDA will deny approval of the PMA or issue a not approvable letter. A not approvable letter will outline the deficiencies in the application and, where practical, will identify what is necessary to make the PMA approvable. The FDA may also determine that additional clinical trials are necessary, in which case the PMA approval may be delayed for several months or years while the trials are conducted and then the data submitted in an amendment to the PMA. Once granted, PMA approval may be withdrawn by the FDA if compliance with post approval requirements, conditions of approval or other regulatory standards is not maintained or problems are identified following initial marketing.

We and our third-party collaborators who may develop our companion diagnostics will work cooperatively to generate the data required for submission with the PMA application, and will remain in close contact with the CDRH to ensure that any changes in requirements are incorporated into the development plans. We anticipate that, as was the case in our meetings to date, future meetings with the FDA with regard to MYDICAR and its companion diagnostic product candidate will include representatives from both CBER and CDRH to ensure that the BLA and PMA submissions are coordinated to enable the FDA to conduct a parallel review of both submissions. On August 6, 2014, the FDA issued a guidance document addressing the development and approval process for "In Vitro Companion Diagnostic Devices." According to the guidance, for novel products such as MYDICAR, the PMA for a companion diagnostic device should be developed and approved contemporaneously with the biological product. We believe our programs for the development of our companion diagnostics are consistent with the guidance. On April 23, 2014, the FDA issued for comment a draft guidance document proposing a new, voluntary program for certain medical devices that demonstrate the potential to address unmet medical needs for life threatening or irreversibly debilitating diseases or conditions that are subject to PMA applications, referred to as the Expedited Access PMA or EAP program. The draft guidance includes references to companion diagnostics as potentially being eligible for the EAP program if the stated criteria are satisfied. The proposed program is designed to help patients have more timely access to these medical devices by expediting their development, assessment and review, while preserving the statutory standard of reasonable assurance of safety and effectiveness for premarket approval. For example, as part of the proposed EAP program, on a case-by-case basis, the FDA may, where appropriate, allow a sponsor to provide less manufacturing information in their PMA application. While this draft guidance is not yet finalized, we believe that the review and approval of our companion diagnostic may qualify for and benefit from elements of the EAP program if the program is ultimately implemented as proposed.

Post-approval Requirements

Maintaining substantial compliance with applicable federal, state and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to GMP. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the GMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products include reporting of GMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency and effectiveness of biological products.

We also must comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMPs and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain GMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

Coverage and Reimbursement

Sales of our products, when and if approved for marketing, will depend, in part, on the extent to which our products will be covered and reimbursed by third-party payors, such as federal, state and foreign government healthcare programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly reducing reimbursements for medical products and services. In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. Decreases in third-party reimbursement for our product candidates or a decision by a third-party payor to restrict access or not cover our product candidates could reduce physician usage of our products once approved and have a material adverse effect on our sales, results of operations and financial condition.

Other Healthcare Laws

Although we currently do not have any products on the market, we may be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. Such laws include, without limitation, state and federal anti-kickback, false claims, privacy and security and physician sunshine laws and regulations. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, disgorgement, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act, to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity.

Government Regulation Outside of the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the European Union, for example, a CTA must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and the IRB, respectively. Once the CTA is approved in accordance with a country's requirements, clinical trial development may proceed.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational biological product under European Union regulatory systems, we must submit a marketing authorization application. The application used to file the BLA in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements. The European Union also provides opportunities for market exclusivity. For example, in the European Union, upon receiving marketing authorization, new chemical entities generally receive eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic product can be marketed until the expiration of the market exclusivity. However, there is no guarantee that a product will be considered by the European Union's regulatory authorities to be a new chemical entity, and products may not qualify for data exclusivity.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Research and Development Expenses

Our research and development expenses were $22.7 million, $16.9 million and $13.3 million for the years ended December 31, 2014, 2013 and 2012, respectively.

Employees

As of March 15, 2015, we had 34 full-time employees, consisting of research, development, manufacturing, finance, legal, administration and business development personnel. We also regularly use independent contractors across the organization to augment our regular staff. None of our employees are covered by collective bargaining agreements and we consider relations with our employees to be good. We believe that our future success will depend in part on our continued ability to attract, hire and retain qualified personnel.

Corporate Information

We were originally incorporated in California in December 2000. In April 2012, we reincorporated in Delaware. Our principal executive offices are located at 11988 El Camino Real, Suite 650, San Diego, California 92130, and our telephone number is (858) 366-4288. Our corporate website address is www.celladon.com. Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and amendments to reports filed pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, will be made available free of charge on our website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The contents of our website are not incorporated into this Annual Report and our reference to the URL for our website is intended to be an inactive textual reference only.

We have obtained a registered trademark for MYDICAR® in the United States. This Annual Report on Form 10-K contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this Annual Report on Form 10-K, including logos, artwork and other visual displays, may appear without the ® or TM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other company.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of our initial public offering in February 2014, (b) in which we have total annual gross revenue of at least $1.0 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeded $700.0 million as of the prior June 30th , and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period. We refer to the Jumpstart Our Business Startups Act of 2012 in this Annual Report on Form 10-K as the "JOBS Act," and references to "emerging growth company" have the meaning associated with it in the JOBS Act.

Unless the context requires otherwise, references to "Celladon," "we," "us" and "our" refer to Celladon Corporation.

Item 1A. Risk Factors

You should carefully consider the following risk factors, as well as the other information in this report, and in our other public filings. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risks Related to our Financial Condition and Capital Requirements

We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a clinical-stage biotechnology company and we have not yet generated any revenues. We have incurred net losses in each year since our inception in December 2000, including consolidated net losses of $33.9 million for the year ended December 31, 2014. As of December 31, 2014, we had an accumulated deficit of approximately $146.4 million. Our prior losses, combined with expected future losses, have had and may continue to have an adverse effect on our stockholders' equity and working capital.

We have devoted most of our financial resources to research and development, including developing our manufacturing capabilities and preclinical and clinical development activities. To date, we have financed our operations primarily through the sale of equity securities and convertible debt. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings or strategic collaborations. We have not completed pivotal clinical trials for any product candidate and it will be several years, if ever, before we have a product candidate ready for commercialization. Even if we obtain regulatory approval to market a product candidate, our future revenues will depend upon the size of any markets in which our product candidates have received approval, and our ability to achieve sufficient market acceptance, reimbursement from third-party payors and adequate market share for our product candidates in those markets.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- initiate, expand or accelerate preclinical and clinical development activities for our lead product candidate, MYDICAR, including with respect to clinical trials of MYDICAR for heart failure for reduced ejection fraction or HFrEF (also referred to as systolic heart failure). Ongoing and planned clinical trials of MYDICAR for HFrEF include CUPID 2, CUPID 3, the LVAD trial, the AAV1 NAb positive trial, the viral shedding trial and a higher dose trial. If supported by data, we also plan to conduct preclinical and potentially clinical activities to evaluate MYDICAR for the treatment of AVF maturation failure, and clinical trials of MYDICAR for the treatment of heart failure for preserved ejection fraction, or HFpEF, (also referred to as diastolic heart failure) and other indications such as PAH;
- further validate and develop the manufacturing process for MYDICAR and our companion diagnostic, including commercial scale-up and contract for the construction and operation of one or more commercial manufacturing facilities, and validate and develop manufacturing processes for our other product candidates and any related companion diagnostics;
- advance our additional preclinical assets, including mSCF gene therapy and our small molecule platform targeting SERCA2 enzymes;
- continue our research and preclinical development of our product candidates and seek to identify and validate additional product candidates;
- seek regulatory and marketing approvals for MYDICAR and its companion diagnostic and any other product candidate that successfully completes clinical trials;

- establish a sales, marketing and distribution infrastructure in the United States to commercialize any products for which we obtain marketing approval;
- acquire rights to other product candidates and technologies;
- change or add manufacturers or suppliers;
- maintain, expand and protect our intellectual property portfolio;
- make milestone or other payments under any in-license or collaboration agreement;
- attract and retain skilled personnel;
- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with any of the above.

The net losses we incur may fluctuate significantly from quarter-to-quarter and year-to-year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

We have never generated any revenue from product sales and may never be profitable.

Our ability to generate meaningful revenue and achieve profitability depends on our ability, and the ability of any third party with which we may partner, to successfully complete the development of, and obtain the regulatory approvals necessary to, commercialize our product candidates and any related companion diagnostics. We do not anticipate generating revenues from product sales for the foreseeable future, if ever. If any of our product candidates fail in clinical trials or if any of our product candidates or any related companion diagnostics do not gain regulatory approval, or if any of our product candidates and any related companion diagnostics, if approved, fail to achieve market acceptance, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our ability to generate future revenues from product sales depends heavily on our success in:

- completing research and preclinical and clinical development of our product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical trials;
- developing a sustainable, scalable, reproducible, and transferable manufacturing process for our vectors and product candidates;
- automating, validating and seeking and obtaining regulatory approvals for our companion diagnostic on a timely basis;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and, if approved, the market demand for our product candidates;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either by establishing a sales force, marketing and distribution infrastructure, or by collaborating with a partner;
- obtaining market acceptance of any approved products and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- identifying and validating new product candidates;

- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product. Our expenses could increase beyond expectations if we are required by the FDA, the EMA, or other foreign regulatory authorities to perform clinical trials and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

Failure to comply with covenants in our existing loan agreement or satisfy certain conditions of the loan agreement, could harm our liquidity, financial condition, business, operating results and prospects.

Under our loan and security agreement with Hercules Technology Growth Capital, Inc. and its affiliate Hercules Technology III, L.P., which we refer to collectively as Hercules or the Lenders, in August 2014 we borrowed $10.0 million from the Lenders and we have the option to borrow up to $15.0 million through June 30, 2015, subject to the satisfaction of certain funding conditions related to our clinical development of MYDICAR. The loan agreement requires us to comply with restrictive covenants, including restrictive covenants that limit our ability to incur additional indebtedness; encumber the collateral securing the loan agreement; acquire, own or make investments; repurchase or redeem stock or other equity securities; declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest; transfer a material portion of our assets; acquire other businesses; or merge or consolidate with or into any other business organization. If we are unable to satisfy the conditions to borrow additional amounts, we may not be able to draw-down additional funds from the loan agreement. Moreover, an uncured breach of any of the covenants or other event of default under the loan agreement could lead to an event of default under the loan agreement. If any event of default occurs, then outstanding amounts under the loan agreement may become due and payable immediately, but we may not have access to such amounts on reasonable terms or at all, which could harm our liquidity, business, financial condition, operating results and prospects.

If we enter into additional debt or credit financing arrangements with the consent of our existing lenders, the terms of such additional debt or credit arrangements could further restrict our operating and financial flexibility. In the event we must cease operations and liquidate our assets, the rights of our existing lenders and any other holder of our outstanding debt would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation.

We will need to raise substantial additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts, preparation for commercial manufacturing or other operations.

We are currently advancing our lead product candidate, MYDICAR for the treatment of HFrEF, through clinical development and other product candidates through preclinical development. Developing products is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates in clinical trials and develop our commercial manufacturing capabilities. We are also entering into contracts with commercial manufacturers relating to the construction and operation of commercial manufacturing facilities. These contracts require substantial financial commitments from us if we choose to move forward with construction, with large payments due prior to any potential product revenue. We expect our expenses to increase substantially if and as we prepare for the commercial manufacturing and potential commercial launch of MYDICAR.

Our operations have consumed substantial amounts of cash since inception. As of December 31, 2014, our cash, cash equivalents and investments were $84.9 million. Our research and development expenses were $22.7 million, $16.9 million and 13.3 million for the years ended December 31, 2014, 2013 and 2012, respectively. In 2015, we plan to initiate additional clinical trials of MYDICAR for HFrEF. In addition, we may develop MYDICAR for additional indications including treatment of AVF maturation failure and for the treatment of patients with advanced heart failure who are on an LVAD. In addition, we are exploring the feasibility of using plasma exchange in removing AAV1 NAbs in advanced heart failure patients prior to administration of MYDICAR. Also, we may initiate a clinical trial in 2016 for the treatment of HFpEF, a condition caused by the inability of the heart to relax normally between contractions, if supported by the CUPID 2 results. We believe that our existing cash, cash equivalents and investments will enable us to fund our operations for at least the next 12 months. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates and companion diagnostic, as well as to further develop MYDICAR for additional indications. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic goals that require additional capital.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates and any related companion diagnostics. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all, even if our clinical trials are successful. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than would otherwise be ideal and we may be required to relinquish rights to some of our technologies, product candidates or our companion diagnostic, or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any approved products or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially adversely affect our business, financial condition and results of operations.

Raising additional funds through debt or equity financing could be dilutive and may cause the market price of our common stock to decline.

To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of those securities could result in substantial dilution for our current stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. Furthermore, the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline and existing stockholders may not agree with our financing plans or the terms of such financings.

In order to raise required funds we may choose to enter into one or more collaborations. Such collaborations could require us to give up substantial rights to MYDICAR in the United States and/or outside the United States.

We may choose to enter into one or more collaborations to raise sufficient capital to continue the development of MYDICAR and our other product candidates and prepare for commercial-scale manufacturing and launch of MYDICAR. These collaborations could require us to relinquish substantial rights, potentially including the grant of an exclusive license to make use and sell MYDICAR, to another company.

Risks Related to the Discovery and Development of our Product Candidates and Companion Diagnostic

We are highly dependent on the success of MYDICAR and we may not be able to successfully obtain regulatory or marketing approval for, or successfully commercialize, this product candidate.

To date, we have expended significant time, resources and effort on the development of MYDICAR for the treatment of HFrEF, including conducting preclinical studies and clinical trials. Although we are preparing for the development of MYDICAR for the treatment of HFpEF and AVF and preclinical activities relating to our small molecule product candidates and our stem cell factor gene therapy program, our ability to generate product revenues and to achieve commercial success will initially depend almost entirely on our ability to successfully develop, obtain regulatory approval for and then successfully commercialize MYDICAR for the treatment of HFrEF in the United States and the European Economic Area, or EEA. Before we can market and sell MYDICAR in the United States or foreign jurisdictions, we will need to commence and complete additional clinical trials, manage clinical and manufacturing activities, obtain necessary regulatory approvals from the FDA in the United States, from the EMA in the EEA, and from other foreign regulatory authorities in other jurisdictions for both MYDICAR and its companion diagnostic, obtain commercial manufacturing supply, build a commercial marketing organization or enter into a commercial marketing collaboration with a third party, and in some jurisdictions, obtain reimbursement authorization, among other things. We cannot assure you that we will be able to successfully complete the necessary clinical trials and/or obtain regulatory approvals and sufficient commercial manufacturing supply for MYDICAR or its companion diagnostic. To date, no gene therapy product has ever been approved in the United States. If we do not receive regulatory approvals, our business, prospects, financial condition and results of operations will be adversely affected. Even if we obtain regulatory approvals, we may never generate significant revenues from any commercial sales of MYDICAR. If we fail to successfully commercialize MYDICAR, we may be unable to generate sufficient revenues to sustain and grow our business and our business, prospects, financial condition and results of operations will be adversely affected.

In April 2015, we plan to un-blind the data from our ongoing CUPID 2 trial. If the results of the CUPID 2 trial are not positive, the market price of our common stock could decline significantly. In addition, patients in the CUPID 2 trial are being followed for a total of five years in an extended long-term follow up period, which period will continue through February 2019. Even if the initial un-blinded results from the CUPID 2 trial are viewed positively, it is possible that negative consequences of the treatment could be observed in the future, which could adversely impact the regulatory and commercial prospects of MYDICAR. Any significant set-back regarding, or the failure of, MYDICAR will have a significant negative impact on our business.

MYDICAR is based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. At the moment, no gene therapy product has been approved in the United States and only one gene therapy product has been approved in Europe.

We have primarily concentrated our research and development efforts on our lead product candidate, MYDICAR, for the treatment of HFrEF, and our future success is highly dependent on the successful development of this product candidate. There can be no assurance that any development problems we experience in the future related to our product candidates will not cause significant delays or unanticipated costs, or that such development problems can be solved. In addition, our product development program is dependent on the development and commercialization of a required companion diagnostic by us or by third party collaborators. Companion diagnostics are subject to regulation as medical devices and those diagnostic tools must independently be cleared or approved by the FDA, the EMA or other foreign regulatory authorities before we may commercialize our product candidates. We may also experience delays in finalizing our commercial manufacturing process or transferring that process to commercial partners or producing clinical trial supplies, which may prevent us from completing our clinical trials or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the EMA and other foreign regulatory authorities, and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. For example, the FDA has required us to conduct a safety and efficacy trial of patients with pre-existing NAbs to the AAV-based vectors used by MYDICAR as well as a viral shedding trial to determine the dissemination of our MYDICAR vector particles into the environment. At the moment, no gene therapy product has been approved in the United States and only one gene therapy product, uniQure's Glybera, which received marketing authorization from the EMA in 2012, has been approved in Europe, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or Europe. Prior product candidate approvals by the EMA may not be indicative of what the FDA or EMA may require for MYDICAR approval.

Regulatory requirements governing gene therapy products have changed frequently and may continue to change in the future. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation. Conversely, the FDA can put an investigational new drug application, or IND, on clinical hold even if the RAC has provided a favorable review. Also, before a clinical trial can begin at an NIH-funded institution, that institution's institutional review board, or IRB, and its Institutional Biosafety Committee, or IBC, will have to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA, the EMA or other foreign regulatory authorities to change the requirements for approval of any of our gene therapy-based product candidates.

These regulatory review committees and advisory groups, and the new guidelines they promulgate, may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance the development of our gene therapy product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approvals necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

Failure to successfully validate, commercialize and obtain regulatory approval for our companion diagnostic could delay or prevent commercialization of MYDICAR. Devices used in the administration of MYDICAR may also require labeling changes and result in delays for the commercialization of MYDICAR.

A key element of our strategy is to screen out patients with certain amounts of pre-existing NAbs to the AAV1 viral vector used as an active ingredient in MYDICAR. We have developed a companion diagnostic that will be used to help us better identify those patients who may benefit from treatment with MYDICAR. Although we are currently exploring the feasibility of using a plasma exchange procedure to remove AAV1 NAbs from advanced heart failure patients to enable their treatment with MYDICAR, the FDA may not permit this procedure to be investigated in certain patient populations, and such a procedure may ultimately prove to be unsafe, ineffective or cost prohibitive, and we cannot predict with certainty when, if ever, such a procedure could successfully be implemented on a broad basis or whether such a procedure would be covered for reimbursement by third-party payors. We will be dependent on such companion diagnostic, both during our clinical trials and in connection with any future commercialization of MYDICAR for HFrEF or for other indications. We expect that we will enter into a strategic alliance with a third party for the automation and commercialization of our

companion diagnostic. We and any of our future collaborators may encounter difficulties in developing the companion diagnostic for commercial application, including issues in relation to automation, selectivity/specificity, analytical validation, reproducibility, critical reagents, or clinical validation of such companion diagnostic. Companion diagnostics are subject to regulation by the FDA as medical devices and require separate regulatory clearance or approval prior to commercialization. In the case of MYDICAR, we anticipate that the FDA will require approval of the companion diagnostic under a medical device pre-market approval, or PMA, application prior to, or concurrently with, approval and commercialization of MYDICAR, which could delay our ability to commercialize both products. If we, or any of our future collaborators, fail to obtain regulatory approval of the companion diagnostic or are delayed in receiving such approval, our ability to commercialize MYDICAR would be delayed until such time as regulatory approval is obtained. In addition, our future collaborators may encounter production difficulties that could constrain the supply of the companion diagnostic, and both they and we may have difficulties gaining acceptance of the use of the companion diagnostic in the clinical community. MYDICAR and certain of the off-the-shelf legally marketed administration components used in the cardiac catheterization laboratory may be regulated as combination products. These include, but are not limited to, regulated products where two or more separate products are packaged together (e.g., drug and device products); or a product packaged separately but intended for use only with an approved, individually specified product where both are required to achieve the intended use of the proposed product. We are in discussions with FDA regarding pre-commercial testing, packaging and labeling requirements for the administration devices and it is possible that MYDICAR may include labeling that specifies certain administration products or product attributes, and the labeling of some of the administration products may need to be changed, e.g., to reflect a change in intended use, which revisions could delay our ability to commercialize MYDICAR.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent development of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit patients to participate in testing our product candidates as well as completion of required follow-up periods. If patients are unwilling to participate in our gene therapy trials because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting trials and obtaining regulatory approval of potential products may be delayed. In addition, if there are delays in accumulating the required number of clinical events in trials where clinical events are a primary endpoint, there may be delays in completing the trial. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the clinical trials altogether.

Patient enrollment and completion of clinical trials are affected by factors including:

- availability of clinical supply of product;
- ability to perform testing for AAV1 NAbs;
- severity of the disease under investigation;
- design of the trial protocol;
- size of the patient population;
- eligibility criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;

- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- ability to monitor patients adequately during and after treatment; and
- the degree of treatment effect in event-driven trials.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics or to complete our clinical trials in a timely manner. We could experience difficulties enrolling the requisite number of patients for future clinical trials, including additional trials that may be required by the FDA for the approval of MYDICAR. For example, one significant obstacle to the timely recruitment and enrollment of a sufficient number of eligible patients in a Phase 3 trial of MYDICAR, if required, is the high prevalence of certain pre-existing NAbs to the viral vector used by MYDICAR, with, we believe, approximately 60% of potential patients in the United States exhibiting these antibodies. In other countries, such as Poland, the prevalence of pre-existing AAV1 NAbs is significantly higher. These antibodies neutralize the effectiveness of AAV-based vectors, such as MYDICAR, and although we are able to prescreen for the presence of these antibodies, the high prevalence of these antibodies in humans reduces the pool of available trial participants.

We plan to seek initial marketing approval for our product candidates in the United States and the EEA. We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA, the EMA or other foreign regulatory authorities. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;
- different standards for conducting clinical trials;
- our inability to locate qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatments.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business.

We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in raising, or inability to raise, sufficient capital to fund the planned clinical trials;
- delays in reaching a consensus with regulatory agencies on trial design;
- delays due to our inability to manufacture and deliver clinical supplies of drug product in a timely fashion;
- identifying, recruiting and training suitable clinical investigators;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;

- delays in obtaining required IRB and IBC approval at each clinical trial site;
- delays in recruiting suitable patients to participate in our clinical trials;
- delays due to changing standard of care for the diseases we are studying;
- delays in dosing or other delays in our clinical trial plans or planned clinical trials as a result of direction from one or more independent data monitoring committees;
- adding new clinical trial sites;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical trial operations or trial sites;
- failure by our CROs, other third parties or us to adhere to clinical trial requirements;
- catastrophic loss of product due to shipping delays or delays in customs in connection with delivery to foreign countries for use in clinical trials;
- failure to perform in accordance with the FDA's good clinical practices, or GCPs, or applicable regulatory guidelines in other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;
- delays in the validation and automation of critical companion diagnostics;
- delays in the manufacture of critical reagents used in any companion diagnostic;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols or supporting information.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. Even though we received Fast Track designation in December 2011 and Breakthrough Therapy designation in April 2014 from the FDA for MYDICAR for the treatment of HFrEF in NYHA Class III/IV heart failure patients, these designations may not result in faster review or approval, if at all. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

If the results of our clinical trials are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates or critical companion diagnostics, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;

- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Our product candidates could potentially cause other adverse events that have not yet been predicted. As described above, any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our products.

Success in early clinical trials may not be indicative of results obtained in later trials.

Trial designs and results from previous trials, including the results from our CUPID 1 and ongoing CUPID 2 trial, are not necessarily predictive of our future clinical trial designs or results. In addition, our CUPID 1 and CUPID 2 trials had a combined enrollment of 301 patients, which we expect may not be a sufficiently high enough number to obtain regulatory approval in the United States or one or more other jurisdictions. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical trials.

There is a high failure rate for drugs and biological products proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical trials even after achieving promising results in earlier stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

The results from our CUPID 2 trial may not be sufficiently robust to support the submission of marketing approval for MYDICAR for the treatment of HFrEF and additional trials may be required to support regulatory approval.

Our ongoing CUPID 2 trial, which is a 250-patient, double-blind, placebo-controlled, randomized Phase 2b clinical trial to evaluate the safety and efficacy of MYDICAR to reduce the frequency of, and/or delay of, heart failure-related hospitalizations in patients with HFrEF, may not be deemed to be a pivotal trial or may not provide sufficient support for a BLA or MAA submission. Although our CUPID 1 trial met its primary safety and efficacy endpoints at six months for high-dose MYDICAR versus placebo and the safety profile from this trial was very favorable, it is still possible that, even if we achieve favorable results in the CUPID 2 trial, the FDA may require us to conduct one or more additional clinical trials, possibly involving a larger sample size or a different clinical trial design, particularly if the FDA does not find the results from the CUPID 2 trial to be sufficiently persuasive to support a BLA submission. For example, the FDA advised us in October 2013 that the number of subjects in our proposed safety database may be an issue to be considered in review of our BLA submission. In addition, the FDA may not accept the use of the mITT analysis population for the CUPID 2 primary analysis of the primary and secondary efficacy endpoints.

In November 2013, the EMA indicated that if MYDICAR demonstrates a substantial and highly significant treatment effect in the advanced heart failure population, and no untoward effects attributable to MYDICAR are observed, a safety database of approximately 205 to 230 MYDICAR-treated subjects may be sufficient to assess safety and allow acceptance of an MAA for MYDICAR for the treatment of HFrEF. However, the EMA has recently indicated that it is not satisfied that adequate control of type 1 error (false positive rate) by the joint

frailty model to be used for the primary efficacy analysis has been demonstrated, and as such, may not consider the results of our CUPID 2 trial to be sufficient for approval of MYDICAR for the treatment of HFrEF. If the FDA or the EMA requires additional studies, we will incur additional costs and delays in the marketing approval process. Even if additional studies are not required for regulatory approval, we will need to expend more resources than we currently have available in order to commercialize MYDICAR. In addition, it is possible that the FDA and the EMA may have divergent opinions on the elements necessary for a successful BLA and MAA, respectively, which may cause us to alter our development, regulatory and/or commercialization strategies.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following any potential marketing approval.

As with many pharmaceutical and biological products, treatment with MYDICAR may produce undesirable side effects or adverse reactions or events. Although extensive preclinical safety and biodistribution testing conducted on MYDICAR and other AAV vectors, including the CUPID 1 trial of MYDICAR for HFrEF and data from previous clinical trials of other AAV vectors, suggests that MYDICAR will be well tolerated, known adverse side effects that could present with treatment with AAV vectors include an immunologic reaction to the capsid protein or gene at early time points after administration. In previous clinical trials involving AAV viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of a T-cell mediated immune response against the vector capsid proteins. As we make plans to initiate a phase 1/2 MYDICAR trial at a dose 2.5-fold higher than previously studied, a T-cell mediated immune response could become more of a risk. If our vectors demonstrate a similar effect, or other adverse events, we may be required to halt or delay further clinical development of our product candidates. In addition, theoretical adverse side effects of AAV vectors include replication and spread of the virus to other parts of the body and insertional oncogenesis, which is the process whereby the insertion of a gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation or cancer. Potential procedure-related events are similar to those associated with standard coronary diagnostic procedures, and may include vascular injury (e.g., damage to the femoral, radial, or brachial arteries at the site of vascular access, or damage to the coronary arteries) or myocardial injury. If any such adverse events occur, our clinical trials could be suspended or terminated and the FDA, the EMA or other foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. The product-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate or the approval may be for a more narrow indication than we expect.

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate and, if applicable, its companion diagnostic, as is the case with MYDICAR. Even if our product candidates demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA advisory committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical trials and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

Even if we obtain regulatory approval in a jurisdiction, regulatory authorities may still impose significant restrictions on the indicated uses or marketing of our product candidates, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with good manufacturing practices, or GMP, and adherence to commitments made in the BLA. If we or a regulatory agency discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory agency may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or supplements to a BLA submitted by us;
- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenues.

Public opinion and heightened regulatory scrutiny of gene therapy and genetic research may impact public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology, with no gene therapy product approved to date in the United States and only one gene therapy product approved to date in Europe. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or, with respect to MYDICAR, in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion could have an adverse effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, in conducting clinical trials of MYDICAR in Europe, we are subject to environmental assessment legislation applicable to genetically modified organisms, or GMOs, which classifies the administration of GMOs to humans

as a "deliberate release" of the GMO into the environment, thereby necessitating prior review and clearance by the applicable environmental assessment governing body. The level of scrutiny varies by country and some localities have additional requirements. Adverse events in our or others' gene therapy clinical trials, even if not ultimately attributable to the product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

Even if we obtain and maintain approval for MYDICAR from one regulatory authority, we may never obtain approval for MYDICAR from regulatory authorities in other jurisdictions, which would limit our market opportunities and adversely affect our business.

Approval in the United States by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of MYDICAR outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries must also approve the manufacturing and marketing of the product candidates in those countries and, if applicable, any required companion diagnostic. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. While we may decide to submit an MAA to the EMA for approval in the EEA, obtaining such approval is a lengthy and expensive process and the EMA has its own procedures for approval of product candidates. Even if a product is approved, the FDA or the EMA, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the EEA also have requirements for approval of drug candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries and regulatory approval in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of MYDICAR will be harmed and our business will be adversely affected.

If approved, MYDICAR or any future products may cause or contribute to adverse medical events that we are required to report to regulatory agencies and if we fail to do so, we could be subject to sanctions that would materially harm our business.

If we are successful in commercializing MYDICAR or any other products, FDA and foreign regulatory agency regulations require that we report certain information about adverse medical events if those products may have caused or contributed to those adverse events. The timing of our obligation to report would be triggered by the date we become aware of the adverse event as well as the nature of the event. We may fail to report adverse events we become aware of within the prescribed timeframe. We may also fail to appreciate that we have become aware of a reportable adverse event, especially if it is not reported to us as an adverse event or if it is an adverse event that is unexpected or removed in time from the use of our products. If we fail to comply with our reporting obligations, the FDA, the EMA or other foreign regulatory authorities could take action including criminal prosecution, the imposition of civil monetary penalties, seizure of our products, or delay in approval or clearance of future products.

Although we have obtained an SPA for a potential Phase 3 pivotal clinical trial of MYDICAR for the treatment of HFrEF, this agreement does not guarantee any particular outcome from regulatory review.

In May 2012, we obtained an SPA from the FDA for a potential Phase 3 pivotal clinical trial of MYDICAR. The FDA's SPA process creates a written agreement between the sponsoring company and the FDA regarding clinical trial design and other clinical trial issues, such as the trial endpoints, that can be used to support approval of a product candidate. The SPA is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of an NDA or BLA. However, an SPA is not a guarantee of an approval of a product candidate or any permissible claims about the product candidate. In particular, an SPA agreement is not binding on the FDA if previously unrecognized public health concerns arise during the performance of the clinical trial, if other new scientific concerns regarding product candidate safety or efficacy arise or if the sponsoring company fails to comply with the agreed upon clinical trial protocols. Moreover, an SPA does not address all of the variables and details that may go into planning for or conducting a clinical trial, and changes in the protocol for a clinical trial can invalidate an SPA or require that the FDA agree in writing to the modified protocol. In addition, while an SPA addresses the requirements for submission of a BLA, the results of the related clinical trial may not support FDA approval.

CUPID 2 is similar in design to the SPA Phase 3 protocol and uses the same primary efficacy endpoint, except that CUPID 2 will use an mITT approach for the primary analysis population as opposed to an intent to treat approach and has a significantly smaller number of subjects. However, we believe that the FDA's agreement of the SPA regarding the trial endpoint is relevant to the CUPID 2 trial. Experience from the CUPID 2 trial has informed the design of a Phase 3 trial and we believe that significant changes to the protocol approved under the SPA are required, which would necessitate written agreement by FDA to ensure binding agreement of the protocol.

Risks Related to our Reliance on Third Parties

We rely on third parties to conduct some or all aspects of our vector production, product manufacturing, combination product commercial supply, companion diagnostic testing, reagent manufacturing, protocol development, research, and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not currently, and do not expect to in the future, to independently conduct all aspects of our vector production, product manufacturing, combination product component supply, companion diagnostic testing, reagent manufacturing, protocol development, research and monitoring and management of our ongoing preclinical and clinical programs. We currently rely, and expect to continue to rely, on third parties with respect to these items, and control only certain aspects of their activities.

Most of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, our product candidate or companion diagnostic development activities may be delayed. Our reliance on these third parties for research and development activities, including the conduct of any IND-enabling studies, reduces our control over these activities but does not relieve us of our responsibility to ensure compliance with all required legal, regulatory and scientific standards and any applicable trial protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical trials are conducted in accordance with the trial plan and protocols.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we may be delayed in completing, or unable to complete, the preclinical studies and clinical trials required to support future IND submissions and approval of our product candidates.

We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates and our companion diagnostic for our clinical trials. There are a small number of suppliers for certain capital equipment and raw materials that we use to manufacture our product candidates and our companion diagnostic. Such suppliers may not sell these raw materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these raw materials. Any significant delay in the supply of a product candidate, a diagnostic reagent, or the raw material components thereof for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates or companion diagnostic. If our manufacturers or we are unable to purchase these raw materials after regulatory approval has been obtained for our product candidates or companion diagnostic, our ability to commercially launch and/or generate revenues from the sale of any of our approved products or companion diagnostic would be impaired. Reliance on third-party manufacturers entails exposure to risks to which we would not be subject if we manufactured the product candidates or companion diagnostic ourselves, including:

- we may be unable to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control over the manufacturing process for our product candidates and companion diagnostic as a result of using third-party manufacturers for all aspects of manufacturing activities;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that may be costly or damaging to us or result in delays in the development or commercialization of our product candidates or companion diagnostic; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to delays in the development of our product candidates or companion diagnostic, including delays in our clinical trials, or failure to obtain regulatory approval for our product candidates or companion diagnostic, or it could impact our ability to successfully commercialize our current product candidates, companion diagnostic or any future products. Some of these events could be the basis for FDA or other regulatory action, including injunction, recall, seizure or total or partial suspension of production.

We and our contract manufacturers are subject to significant regulation with respect to manufacturing our viral vectors, product candidates and companion diagnostic. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.

We currently have a relationship with only two suppliers, Lonza and Novasep, for the manufacturing of our viral vectors and product candidates for clinical testing purposes. We expect to rely upon Lonza, Novasep, and/or other third parties to produce materials required for the commercial production of our product candidates and companion diagnostic if we succeed in obtaining the necessary regulatory approvals. Because certain of our license agreements place restrictions on our ability to transfer or sublicense our intellectual property rights obtained under such agreements in connection with manufacturing activities, if any supplier we use requires a sublicense of our intellectual property rights for commercial manufacture of our viral vectors, product candidates or companion diagnostic, we may be unable to transfer or sublicense the requisite intellectual property rights, which may negatively impact our supply of our viral vectors, product candidates or companion diagnostic.

All entities involved in the preparation of therapeutic product for clinical trials or commercial sale, including our existing contract manufacturers, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with GMP and equivalent foreign standards. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and

assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to GMP regulations enforced by the FDA through its facilities inspection program. Any failure by our third-party manufacturers to comply with GMP or failure to scale-up manufacturing processes, including any failure to deliver sufficient quantities of product candidates in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of any of our product candidates or companion diagnostic. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must also pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or companion diagnostic. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates, companion diagnostic or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities and quality systems do not pass a pre-approval plant inspection, FDA approval of our product candidates, or the equivalent approvals in other jurisdictions, will not be granted.

Regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biological product, or revocation of a pre-existing approval. If any such event occurs, our business, financial condition and results of operations may be materially harmed.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA supplement which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching or adding manufacturers may involve substantial costs and would likely result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and if we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

We rely on third parties to conduct, supervise and monitor our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We rely on CROs and clinical trial sites to ensure our clinical trials are conducted properly and on time. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our principal investigators and CROs are required to comply with the FDA's and the ICH's (the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use) GCPs for conducting, recording and reporting the results of clinical trials to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. The FDA, the Competent Authorities of the Member States of the EEA, and comparable foreign regulatory authorities, enforce these GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we, our principal investigators or our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA, or other foreign regulatory authorities may require us to perform additional clinical trials before approving any marketing applications. Upon inspection, the FDA may determine that our clinical trials did not comply with GCPs. In addition, our future clinical trials will require a sufficient number of patients to evaluate the safety and effectiveness of our product candidates. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities that could harm our competitive position. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize, our product candidates. If any such event were to occur, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. Further, switching or adding additional CROs involves additional costs and requires management time and focus. We have recently entered into a contract with a new CRO for new clinical trials. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which could materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

We also rely on other third parties to store and distribute our vectors and products for the clinical trials that we conduct. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, if approved, producing additional losses and depriving us of potential product revenue.

We may seek to form strategic alliances in the future with respect to our product candidates or companion diagnostic, and we may not realize the benefits of such alliances.

We may form strategic alliances, create joint ventures or collaborations or enter into licensing arrangements with third parties for the development and commercialization of our product candidates and companion diagnostic. We face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Any delays in entering into new strategic partnership agreements related to our product candidates or companion diagnostic could delay the development and commercialization of our product candidates and reduce their competitiveness even if they reach the market. Moreover, we may not be successful in our efforts to establish a strategic partnership or other collaborative arrangement for any future product candidates or companion diagnostic because the potential partner may consider that our research and development pipeline is insufficiently developed to justify a collaborative effort, or that our product candidates

and programs do not have the requisite potential to demonstrate safety and efficacy in the target population. Even if we are successful in establishing such a strategic partnership or collaboration, we cannot be certain that, following such a strategic transaction or license, we will be able to progress the development and commercialization of the applicable product candidates as envisaged, or that we will achieve the revenues that would justify such transaction.

Risks Related to Commercialization of our Product Candidates and Companion Diagnostic

We intend to rely on third parties to produce our viral vectors, product candidates and other key materials and for our companion diagnostic testing, but these manufacturers have minimal or no experience producing our vectors, product candidates or companion diagnostic materials at commercial levels and may not achieve the necessary regulatory approvals or produce our vectors, products or companion diagnostic materials at the quality, quantities, locations and timing needed to support commercialization.

We are currently developing a scalable manufacturing process for MYDICAR, which we have transferred to Lonza and are in the process of transferring to Novasep. We have completed a first demonstration batch at commercial scale of production (2,000-liter production bioreactor scale) at Lonza in Houston, TX, and are in the process of testing this material for conformance to our specifications. Although we have entered into an agreement for the manufacture of our MYDICAR drug substance with Lonza for our clinical trials, Lonza may not perform as agreed, may be unable to comply with GMP requirements and with FDA, state and foreign regulatory requirements or may terminate its agreement with us. If Lonza is unable to manufacture MYDICAR drug substance in a timely manner, encounters manufacturing difficulties, or otherwise fails to comply with its contractual obligations and we are required to switch to a new manufacturer, we expect that our clinical development timeline would be delayed by at least one year. Because of the complex nature of our product candidates, Lonza, Novasep, or any other manufacturer with whom we may enter into an agreement, may not be able to manufacture our product candidates at a cost or in quantities or on timelines necessary for the successful commercialization of our product candidates. If we successfully commercialize any of our product candidates, we will be required to establish large-scale commercial manufacturing capabilities, relying on one or more third parties, and there is no guarantee that any such third parties will be able to do this in a timely manner, or at all. In addition, in the event that our product development pipeline increases and matures, we will have a greater need for clinical trial and commercial manufacturing capacity. We have minimal experience manufacturing pharmaceutical or biological products on a commercial scale and our potential suppliers, including Lonza and Novasep, will have to construct and validate new commercial manufacturing facilities and obtain regulatory approvals for the facilities before being able to produce MYDICAR, and there can be no assurance that they will succeed in doing so.

Even if we develop a commercial scale manufacturing process in a timely fashion and successfully transfer it to Lonza, Novasep, or any other third-party vector and product manufacturers, if such third-party manufacturers are unable to produce our viral vectors or product candidates in the necessary quantities, or in compliance with GMP, or in compliance with pertinent regulatory requirements, and within our planned time frame and cost parameters, the development and sales of our products, if approved, may be materially harmed.

We similarly intend to enter into agreements with third parties for the automation, characterization and validation, of our companion diagnostic and the manufacture of its critical reagents. However, we may be unable to enter into such an agreement on favorable terms, or at all.

We may run into technical or scientific issues related to manufacturing or development that we may be unable to resolve in a timely manner or with available funds. In addition, we have not completed the development, characterization and validation activities necessary for commercial and regulatory approvals. If Lonza, Novasep, or any of our other manufacturing partners does not obtain such regulatory approvals for their facilities, our commercialization efforts will be harmed. In addition, any significant disruption in our supplier relationships could harm our business. We source key materials from third parties, either directly through agreements with suppliers or indirectly through our manufacturers who have agreements with suppliers. There

are a small number of suppliers for certain key materials that are used to manufacture our product candidates and companion diagnostic. Such suppliers may not sell these key materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have any control over the process or timing of the acquisition of these key materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these key materials.

Our decision as to whether to exercise the construction trigger under our facility construction and commercial supply agreement with Lonza and/or proceed with the next phase of our agreement with Novasep will depend on a number of factors. If we are unable to or determine not to exercise our rights under either or both agreements, the manufacturer will not proceed with the construction of or validation of their proposed facility and we will need to identify, negotiate terms for and secure alternate sources of commercial supply of MYDICAR in order to commercialize MYDICAR. We may need to decide whether to exercise one or both of these triggers based on incomplete information regarding the potential approvability of MYDICAR or the potential timelines for commercial launch, and each of these triggers entails a very substantial financial commitment by us.

In October 2014, we entered into a Facility Construction and Commercial Supply Agreement with Lonza Biologics, Inc., or Lonza, pursuant to which we and Lonza agreed to initiate detailed design planning for the potential construction of a new commercial viral therapeutics facility in Portsmouth, New Hampshire for the manufacture of MYDICAR drug substance (AAV1/SERCA2a), and in exchange for an upfront reservation fee payable by us to Lonza, Lonza agreed to reserve, for a period of time extendable on payment of specified reservation extension fees, the capital, property and labor resources necessary to enable the initiation of construction of the facility within 75 days of receipt of notice of our decision to initiate construction of the facility and commit to a long-term supply arrangement for MYDICAR, or the construction trigger. The construction trigger may not be exercised by us prior to completion of an agreed upon detailed design for the facility, which we currently expect may be completed during the third quarter of 2015, but there can be no assurance that it will be completed on this timeframe, or at all. Our decision as to whether to exercise the construction trigger may be impacted by a number of factors, including, among others, the outcome of our CUPID 2 trial of MYDICAR, our expectations regarding clinical trial requirements and development timelines, and our perception of the prospects for commercialization of MYDICAR. Even if we view the results of our CUPID 2 trial as sufficiently favorable to support a decision to exercise the construction trigger, our ability to do so may be limited by a number of additional factors, including our ability to obtain financing on terms sufficient to fund our financial obligations under the agreement following exercise of the construction trigger. In addition, under our loan and security agreement with Hercules, we will be required to either obtain the consent of Hercules prior to exercising the construction trigger or pay off our outstanding loan under the agreement, and there is no guarantee that we will be able to do either within the timeframe available to us to exercise the construction trigger.

In March 2015 we entered into a Development, Manufacturing and Supply Agreement with Novasep pursuant to which we agreed to continue process transfer activities to enable Novasep to complete manufacturing scale-up and validation of a facility in Europe. We have the right to terminate this agreement, exercisable for a specified period of time following the un-blinding of the CUPID 2 data, if we conclude in good faith that the CUPID 2 data is such that we do not require production of MYDICAR drug substance at the Novasep Facility. We will need to decide whether to terminate the agreement or proceed with very substantial commitments to pay for the design, construction activities and operation of the manufacturing facility, including the purchase of commercial lots of MYDICAR on a “take or pay” basis, which means that we must commit to a minimum amount of purchases of MYDICAR through the end of 2018 or (or 2019 if we choose to have the facility be dedicated solely to MYDICAR drug substance manufacture) or make significant payments to Novasep. This decision has material consequences for the rate of our expenditures going forward, and will need to be made based upon incomplete information regarding the probability of regulatory approval of MYDICAR and the timeline for potential commercial launch assuming regulatory approval is obtained.

Even if we proceed with one or both of the Lonza or Novasep agreements, we may not realize the anticipated benefits under the agreements and may incur considerable losses and commercialization delays associated with the construction or validation of the facilities. There can be no assurance that the construction or validation of the facilities will be completed within the budget we anticipate or in a timely manner, or in compliance with applicable laws and regulations. In connection with our continuation of the Lonza and/or Novasep agreements, we will be required to commit to one or more long-term supply arrangements that will involve minimum purchase obligations.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates or companion diagnostic, if approved, we may be unable to generate any revenues.

We currently do not have an organization for the sales, marketing and distribution of products and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any products that may be approved, including MYDICAR, we must build our sales, distribution, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We have no prior experience in the marketing, sale or distribution of pharmaceutical or diagnostic products and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain, and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. We will likely seek to enter into strategic partnerships with third parties to commercialize our product candidates and companion diagnostic outside of the United States. We intend to build an internal sales and marketing organization for the commercialization of MYDICAR in the United States. However, we will also consider the option to enter into strategic partnerships for our product candidates and companion diagnostic in the United States and other geographies where we obtain marketing approval. We may need to give up substantial rights to MYDICAR in the United States and outside the United States in order to finance the continued development, manufacturing and commercial launch of MYDICAR.

Our strategy for MYDICAR is to develop a hospital-directed specialty sales force and/or collaborate with third parties to promote the product to selected cardiologists, heart failure specialists, other health care providers and third-party payors in the United States. Some pharmaceutical companies employ groups of sales representatives of much larger scale than we intend to utilize to target their cardiovascular products for the general physician community and third-party payors. We will likely seek to align ourselves with collaborators as part of our commercialization strategy, particularly outside of the United States, and our future collaboration partners, if any, may not dedicate sufficient resources to the commercialization of our product candidates or companion diagnostic or may otherwise fail in their commercialization due to factors beyond our control. If we are unable to establish effective collaborations to enable the sale of our product candidates and companion diagnostic to healthcare professionals and in geographical regions, including the United States, that will not be covered by our own marketing and sales force, or if our potential future collaboration partners do not successfully commercialize our product candidates or companion diagnostic, our ability to generate revenues from product sales, including sales of MYDICAR, will be adversely affected.

Building an internal sales force involves many challenges, including:

- recruiting and retaining talented people;
- training employees who we recruit;
- setting the appropriate system of incentives;
- managing additional headcount; and
- integrating a new business unit into an existing corporate architecture.

If we are unable to build our own sales force or negotiate a strategic partnership for the commercialization of MYDICAR or our companion diagnostic in the United States, we may be forced to delay the potential commercialization of MYDICAR, reduce the scope of our sales or marketing activities for MYDICAR or undertake the commercialization activities for MYDICAR at our own expense. If we elect to increase our expenditures to fund commercialization activities ourselves, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. If we do not have sufficient funds, we will not be able to bring MYDICAR to market or generate product revenue. We could enter into arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to some of our technologies, product candidates or our companion diagnostic or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate sufficient product revenue and may not become profitable. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

In addition, there are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

If the market size for MYDICAR is considerably smaller than we anticipate, it could significantly and negatively impact our business, financial condition and results of operations.

It is very difficult to estimate the future commercial potential of MYDICAR due to factors such as safety and efficacy compared to other available treatments, changing standards of care, achieving favorable hospital formulary status, third-party payor reimbursement standards, ability of patients to meet co-payment amounts, patient and physician preferences, and the availability of competitive alternatives that may emerge. We believe that approximately 60% of such potential patients in the United States will be ineligible for treatment with MYDICAR due to the presence of pre-existing AAV1 NAbs which will neutralize the effectiveness of AAV-based vectors such as MYDICAR. In other countries, such as Poland, the prevalence of pre-existing AAV-resistant antibodies is significantly higher. In addition, just one exposure to an AAV-based treatment such as MYDICAR may cause a patient to produce NAbs. Furthermore, other pharmaceutical companies could develop and receive approval for new AAV-based treatments which could increase the number of patients that exhibit NAbs. We estimate that there are over 350,000 heart failure patients in the United States alone that would be eligible for MYDICAR treatment upon launch, if approved; however, if the potential eligible patient population is lower than we anticipate, or if considerably more than 60% of potential patients exhibit NAbs, it could significantly and negatively impact our business, financial condition and results of operations.

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.

We face competition both in the United States and internationally, including from major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Some of the pharmaceutical and biotechnology companies we know are developing gene therapies for heart failure that could potentially be competitive with or hinder the uptake of MYDICAR and change the standard of care for

heart failure patients include Renova Therapeutics, NanoCor Therapeutics, Juventas Therapeutics, VentriNova, uniQure N.V. and Beat BioTherapeutics. In addition, many universities and private and public research institutes are active in our target disease areas.

Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop, or achieve earlier patent protection, regulatory approval, product commercialization and market penetration than we do. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

Under the terms of our license agreement with AskBio LLC, or AskBio, we granted AskBio an option to obtain a non-exclusive, worldwide license under certain of our patent rights related to infusion of AAV in the arteries of the heart to develop, manufacture, use and sell products for the treatment of cardiac diseases. This option includes our currently pending patent application related to a method of treating a cardiovascular disease by infusion of a therapeutic nucleic acid into the coronary circulation over a specified period of time. It does not include our issued patent in this family, which includes claims to the concurrent use of a vasodilating substance such as nitroglycerin. Although the scope of the license granted to AskBio excludes our issued patent and the scope of our anticipated regulatory approvals, there can be no guarantee AskBio will not seek to develop and commercialize a product that is able to compete with MYDICAR.

Even if we are successful in achieving regulatory approval to commercialize a product candidate faster than our competitors, we may face competition from “biosimilars” due to the changing regulatory environment. In the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biological products that are demonstrated to be “highly similar,” or “biosimilar,” to or “interchangeable” with an FDA-approved biological product. This new pathway could allow competitors to reference data from biological products already approved after 12 years from the time of approval in the United States. In addition, companies may be developing biosimilars in other countries that could compete with our products. If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences. Expiration or successful challenge of our applicable patent rights could also trigger competition from other products, assuming any relevant exclusivity period has expired.

Finally, as a result of the expiration or successful challenge of our patent rights, we could face litigation with respect to the validity and/or scope of patents relating to our competitors’ products. The availability of our competitors’ products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize.

The commercial success of any current or future gene therapy product candidate will depend upon the degree of market acceptance by physicians, patients, third-party payors and other health care providers in the medical community.

Ethical, social and legal concerns about gene therapy and genetic research could result in additional regulations restricting or prohibiting the products and processes we may use. Even with the requisite approvals, the commercial success of our gene therapy product candidates will depend in part on the medical community, patients, and third-party payors accepting gene therapy products in general, and our product candidates in particular, as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors and other health care providers in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant

product revenue and may not become profitable. The degree of market acceptance of these product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the potential efficacy and potential advantages over alternative treatments;
- the clinical indications for which the product candidate is approved;
- with respect to MYDICAR, the approval, availability and market acceptance, coverage and reimbursement for the companion diagnostic;
- ability of patients to pay co-payment amounts, if applicable;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- acceptance by physicians and patients of the product as a safe and effective treatment;
- the potential and perceived advantages of such product candidate over alternative treatments, especially with respect to patient subsets that we are targeting with such product candidate;
- the existence of other gene therapy products utilizing an AAV vector, which potential patients may elect to take for other indications, thereby causing them to develop NAbs and making them ineligible to take MYDICAR;
- the safety of such product candidate seen in a broader patient group, including its use outside the approved indications;
- the cost of treatment in relation to alternative treatments;
- relative convenience and ease of administration;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- the effectiveness of our sales and marketing efforts;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage or reimbursement

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate the medical community and third-party payors on the benefits of the product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors.

If we are unable to achieve and maintain adequate levels of coverage and reimbursement for MYDICAR, our companion diagnostic or any other product candidates, if approved, on reasonable pricing terms, their commercial success may be severely hindered.

Successful sales of any approved product candidates depend on the availability of adequate coverage and reimbursement from third-party payors. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Sales of our product candidates will therefore depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed

care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, such as Medicare and Medicaid, private health coverage insurers and other third-party payors. Co-pay amounts under Medicare (generally 20% of the cost of the treatment for patients without supplemental insurance) or other third-party payor systems may be a substantial hindrance to certain patients' ability to pay for MYDICAR treatment. In addition, the market for MYDICAR and any of our other product candidates will depend significantly on access to third-party payors' formularies, or lists of treatments for which third-party payors provide coverage and reimbursement and patients' ability to make copayments, if applicable.

Third-party payors, whether foreign or domestic, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In the United States, no uniform policy of coverage and reimbursement for therapeutic products exists among third-party payors. Therefore, coverage and reimbursement for therapeutic products can differ significantly from payor to payor. As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that adequate coverage and reimbursement will be obtained. In many foreign countries, a product candidate must be approved for reimbursement before it can be approved for sale in that country, and we may fail to obtain such reimbursement approvals.

In the United States, decisions about Medicare coverage and reimbursement for new medicines are made by the Centers for Medicare & Medicaid Services, or CMS, the agency within the U.S. Department of Health and Human Services responsible for administering the Medicare program. Private payors and other government payors often follow CMS's policies to a substantial degree, making the Medicare determinations particularly significant. It remains uncertain what CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Similarly, outside the United States, we may not succeed in obtaining reimbursement approval from the relevant regulatory authorities.

In addition, coverage decisions may depend upon clinical and economic standards that disfavor new products when more established or lower cost alternatives are already available or subsequently become available. Assuming we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Middle Class Tax Relief and Job Creation Act of 2012 required CMS to reduce the Medicare Clinical Laboratory Fee Schedule, or CLFS, by 2% in 2013, which in turn serves as a base for 2014 and subsequent years. In addition, CMS announced that it will bundle the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting beginning on January 1, 2014.

More recently, on April 1, 2014, the Protecting Access to Medicare Act of 2014, or PAMA, was signed to law, which, among other things, significantly alters the current payment methodology under the CLFS. Under the new law, starting January 1, 2016 and every three years thereafter (or annually in the case of advanced diagnostic lab tests), applicable clinical laboratories must report laboratory test payment data for each Medicare-covered clinical diagnostic lab test that it furnishes during a time period to be defined by future regulations. The reported data must include the payment rate (reflecting all discounts, rebates, coupons and other price concessions) and the volume of each test that was paid by each private payor (including health insurance issuers, group health plans, Medicare Advantage plans and Medicaid managed care organizations). Beginning in 2017, the Medicare payment rate for each clinical diagnostic lab test will be equal to the weighted median amount for the test from the most recent data collection period. The payment rate will apply to clinical laboratory diagnostic tests furnished by a hospital laboratory if the test is separately paid under the hospital outpatient prospective payment system. Additionally, PAMA overrules reforms included in the 2014 final rule for the Medicare Physician Fee Schedule that called for a process to permit CMS to adjust payments under the CLFS to account for technological changes in tools, machines, supplies, labor, instruments, skills, techniques and devices by which laboratory tests are produced and used beginning in 2015. Levels of reimbursement may be impacted by these initiatives and other current and future legislation, regulation or reimbursement policies of third-party payors in a manner that

may harm the demand and reimbursement available for our products, including our companion diagnostic, which in turn, could harm our product pricing and sales.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Third-party coverage and reimbursement for MYDICAR or any of our other product candidates for which we may receive regulatory approval may not be available or adequate in either the United States or international markets and may vary substantially from our current assumptions, which could have a material adverse effect on our business, results of operations, financial condition and prospects.

Healthcare reform measures may have a material adverse effect on our business and results of operations.

In the United States, the legislative landscape continues to evolve. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Affordable Care Act, was passed, which has the potential to substantially change health care financing by both governmental and private insurers, and significantly impact the U.S. pharmaceutical industry. The Affordable Care Act, among other things, subjects biological products to potential competition by lower-cost biosimilars, revised the methodology by which rebates owed by manufacturers for covered outpatient drugs under the Medicaid Drug Rebate Program are calculated, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, and provided incentives to programs that increase the federal government's comparative effectiveness research.

In addition, other legislative changes have been proposed and adopted in the United States since the Affordable Care Act was enacted. On August 2, 2011, the Budget Control Act of 2011 among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013 and will remain in effect through 2024 unless additional Congressional action is taken. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which, among other things, further reduced Medicare payments to certain providers, including physicians, hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions. We expect that we will be subject to additional risks related to entering into international business relationships, including:

- different regulatory requirements for approval of drugs and biological products in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- regulations under the U.S. Foreign Corrupt Practices Act and similar foreign anti-corruption laws;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

Risks Related to our Business Operations

Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on the members of our executive team, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into offer letters with each of our executive officers, any of them could leave our employment at any time, as all of our employees are "at will" employees. Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, will also be critical to our success.

Although we have not historically experienced unique difficulties attracting and retaining qualified employees, we could experience such problems in the future. For example, competition for qualified personnel in the biotechnology and pharmaceuticals field is intense due to the limited number of individuals who possess the skills and experience required by our industry. There is currently a shortage of skilled executives in our industry, which is likely to continue. As a result, competition for skilled personnel is intense and the turnover rate can be high. We will need to hire additional personnel as we expand our clinical development and commercial activities. We may not be able to attract and retain personnel on acceptable terms, or at all, given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical studies or clinical trials may make it more challenging to recruit and retain qualified personnel. The inability to recruit or loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives. Furthermore, to the extent we hire personnel from competitors, we may be subject to allegations that they have been improperly solicited or that they have divulged proprietary or other confidential information, or that their former employers own their research output.

If CUPID 2 data are positive, we will need to expand our organization substantially in a short period of time and we may experience difficulties in managing this growth, which could disrupt our operations.

As of March 15, 2015, we had 34 full-time employees. As we mature and expand our research and development, commercial manufacturing, quality control and quality assurance and other pre-commercialization activities, we expect to expand our full-time employee base and to hire more consultants and contractors. In addition, we currently plan to commercialize MYDICAR, if approved, using an internal sales force to target selected cardiologists, heart failure specialists and third-party payors in the United States. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraudulent conduct or other illegal activity by our employees, independent contractors, principal investigators, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, promotion, sales, marketing and certain business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of patient recruitment or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of business conduct and ethics applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws, including, without limitation, the federal Health Care Program Anti-Kickback Statute and the federal civil and criminal False Claims Acts. These laws may impact, among other things, our proposed promotional, sales, marketing and educational programs. In

addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include, but are not limited to:

- the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind in return for, the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other government payers that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;
- the federal Food, Drug and Cosmetic Act, or FDCA, which prohibits, among other things, the adulteration or misbranding of drugs and devices;
- federal transparency laws, including the federal Physician Payment Sunshine Act that requires certain drug manufacturers to disclose payments and other transfers of value provided to physicians and teaching hospitals, as well as ownership and investment interests held by such physicians and their immediate family members;
- the Affordable Care Act, and its implementing regulations, which may impact, among other things, reimbursement rates by federal health care programs and commercial insurers; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states.

Further, the Affordable Care Act, among other things, amends the intent requirements of the federal Anti-Kickback Statute and the criminal statute governing healthcare fraud. A person or entity can now be found guilty of violating the Anti-Kickback Statute and the federal criminal healthcare fraud statute without actual knowledge of the statute or specific intent to violate it. In addition, the Affordable Care Act provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act.

If our operations are found to be in violation of any of the health regulatory laws described above or any other laws that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in federal health care programs and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our financial results.

We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use or misuse of our product candidates harms patients, or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.

The use or misuse of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation;
- initiation of investigations by regulators;
- withdrawal of clinical trial participants;
- costs due to related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates;
- product recalls, withdrawals or labeling, marketing or promotional restrictions; and
- decreased demand for our product candidates, if approved for commercial sale.

We carry product liability insurance of $10.0 million per occurrence and a $10.0 million aggregate limit. We believe our product liability insurance coverage is appropriate in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Patients with the diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may or may not be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions. We do not currently carry biological or hazardous waste insurance coverage.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities.

We may not be successful in our efforts to identify or discover additional product candidates.

The success of our business depends primarily upon our ability to identify, develop and commercialize products based on our gene therapy, stem cell factor and small molecule platforms. Our research programs may fail to identify other potential product candidates for clinical development for a number of reasons. For example, our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which may have a material adverse effect on our business and could potentially cause us to cease operations. Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or

target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. The recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the recent global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and a decreased ability to raise additional capital when needed on acceptable terms, if at all. This is particularly true in Europe, which is undergoing a continued severe economic crisis. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Our business and operations would suffer in the event of system failures.

Despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contractors, consultants and potential collaborators are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on third parties to manufacture our vectors, our product candidates and our companion diagnostic and conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates and any related companion diagnostics could be delayed.

Interruptions in the supply of product or inventory loss may adversely affect our operating results and financial condition.

Our product candidates and companion diagnostic are manufactured and distributed using technically complex processes requiring specialized facilities, highly specific raw materials and other production constraints. The complexity of these processes, as well as strict company and government standards for the manufacture and storage of our products, subjects us to production risks. While product batches released for use in clinical trials or for commercialization undergo sample testing, some defects may only be identified following product release. In addition, process deviations or unanticipated effects of approved process changes may result in these intermediate products not complying with stability requirements or specifications. Most of our product candidates must be stored and transported at temperatures within a certain range. If these environmental conditions deviate, our product candidates' remaining shelf-lives could be impaired or their efficacy and safety could become adversely affected, making them no longer suitable for use. The occurrence or suspected occurrence of production and distribution difficulties can lead to lost inventories, and in some cases product recalls, with consequential reputational damage and the risk of product liability. The investigation and remediation of any identified problems can cause production delays, substantial expense, lost sales and delays of new product launches. Any interruption in the supply of finished products or the loss thereof could hinder our

ability to timely distribute our products and satisfy customer demand. Any unforeseen failure in the storage of the product or loss in supply could delay our clinical trials and, if our product candidates are approved, result in a loss of our market share and negatively affect our revenues and operations.

We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. A majority of our management operates in our principal executive offices located in San Diego, California. If our San Diego offices were affected by a natural or man-made disaster, particularly those that are characteristic of the region, such as wildfires and earthquakes, or other business interruption, our ability to manage our domestic and foreign operations could be impaired, which could materially and adversely affect our results of operations and financial condition. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business. We currently rely, and intend to rely in the future, on our third-party manufacturer, Lonza, to produce our clinical supply of MYDICAR. Our ability to obtain supply of MYDICAR could be disrupted, and our results of operations and financial condition could be materially and adversely affected if the operations of Lonza were affected by a man-made or natural disaster or other business interruption. The ultimate impact of any such events on us, our significant suppliers and our general infrastructure is unknown. For more information regarding our manufacturing services agreement with Lonza, see “Business—Manufacturing—Manufacturing Services Agreement with Lonza” in this Annual Report.

Risks Related to our Intellectual Property

If we are unable to obtain or protect intellectual property rights related to our product candidates and companion diagnostic, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates and companion diagnostic. The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates and companion diagnostic in the United States or in other foreign countries. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our product candidates or companion diagnostic, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates or companion diagnostic or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

If the patent applications we hold or have in-licensed with respect to our programs, product candidates and companion diagnostic fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates or companion diagnostic, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products.

Several patent applications covering our product candidates and companion diagnostic have been filed recently. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates or companion diagnostic that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate and companion diagnostic under patent protection could be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we were the first to file any patent application related to a product candidate or companion diagnostic. Furthermore, if third parties have filed such patent applications, an interference proceeding in the United States can be initiated by a third party to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. In addition, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates and companion diagnostic are obtained, once the patent life has expired for a product, we may be open to competition from generic medications.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidates and companion diagnostic discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with those who have access to our confidential information, including our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted rights to intellectual property that are important to our business and we expect that we may need to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose on us, various development, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license. Additionally, several of our existing license agreements are sublicenses from a third party who is not the original licensor of the intellectual property at issue. Under these agreements, we must rely on our licensor to comply with their obligations under the primary license agreements under which such third party obtained rights in the applicable intellectual property, where we may have no relationship with the original licensor of such rights. If our licensors fail to comply with their obligations under these upstream license agreements, the original third-party licensor may have the right to terminate the original license, which may terminate our sublicense. If this were to occur, we would no longer have rights to the applicable intellectual property unless we are able to secure our own direct license with the owner of the relevant rights, which we may not be able to do at a reasonable cost or on reasonable terms, which may impact our ability to continue to develop and commercialize our product candidates and companion diagnostic incorporating the relevant intellectual property. See "Business—License Agreements" in this Annual Report for a description of our license agreements, which includes a description of the termination provisions of these agreements.

As we have done previously, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates or companion diagnostic, and we cannot provide any assurances that third-party patents do not exist which might be enforced against our current product candidates or companion diagnostic or future products in the absence of such a license. We may fail to obtain any of these licenses on commercially reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates or companion diagnostic, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates or the companion diagnostic, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates or any related companion diagnostics.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* review proceedings before the U.S. Patent and Trademark Office, or U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates and our companion diagnostic. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates and companion diagnostic may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates and companion diagnostic. Because patent applications can take many years to issue, third parties may have currently pending patent applications which may later result in issued patents that our product candidates or companion diagnostic may infringe, or which such third parties claim are infringed by the use of our technologies. If any third-party patents are held by a court of competent jurisdiction to cover any aspect of the manufacturing process for any of our product candidates or companion diagnostic, any molecules formed during the manufacturing process, or any final product candidate or companion diagnostic, including the formulation or method of use of such product candidate or companion diagnostic, the holders of any such patents may be able to block our ability to commercialize such product candidate or companion diagnostic unless we obtained a license under the applicable patents, or until such patents expire. In any such case, such a license may not be available on commercially reasonable terms or at all.

Parties making claims against us for infringement of their intellectual property rights may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates or any related companion diagnostics. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we could be required to redesign our infringing products or obtain a license from such third party to continue developing and commercializing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. It may be impossible to redesign our products and technology, or it may require substantial time and monetary expenditure, which could force us to cease commercialization of one or more of our product candidates or the companion diagnostic, or some of our business operations, which could materially harm our business. In addition, in any such proceeding, we may be required to pay substantial damages, including treble damages and attorneys' fees in the event we are found liable for willful infringement.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently we have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our product candidates and companion diagnostic. Because our programs may involve additional product candidates or companion diagnostics that may require the use of proprietary rights held by

third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates and companion diagnostic may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates and companion diagnostic. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us, either on reasonable terms, or at all. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment, or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights, our ability to commercialize our products, and our business, financial condition and prospects for growth could suffer.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. A third-party defendant may also request post grant review or *inter partes* review by the U.S. PTO of any patent we assert. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

The patent protection and patent prosecution for some of our product candidates and companion diagnostic may be dependent on third parties.

While we normally seek to obtain the right to control the prosecution and maintenance of the patents relating to our product candidates and companion diagnostic, there may be times when the filing and prosecution activities for platform technology patents that relate to our product candidates and companion diagnostic are controlled by our licensors. For example, we do not have the right to prosecute and maintain the patent rights licensed to us under agreements with each of The Regents of the University of California, AmpliPhi (including the patent rights sublicensed to us from UPenn), Virovek Incorporation, AskBio and Dr. Martin J. Kaplitt, and our ability to have input into such filing and prosecution activities is limited. If these licensors or any of our future licensors fail to appropriately prosecute and maintain patent protection for patents covering any of our product candidates or companion diagnostic, our ability to develop and commercialize those product candidates and companion diagnostic may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to manufacture our vectors, product candidates and companion diagnostic, and because we collaborate with various organizations and academic institutions on the advancement of our gene therapy and small molecule platforms and companion diagnostic, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. For example, any academic institution that we may collaborate with in the future will usually expect to be granted rights to publish data arising out of such collaboration, provided that we are notified in advance and given the opportunity to delay publication for a limited time period in order for us to secure patent protection of intellectual property rights arising from the collaboration, in addition to the opportunity to remove confidential or trade secret information from any such publication. In the future we may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

In September 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The U.S. PTO is currently developing regulations and procedures to govern administration of the Leahy-Smith Act, and many of

the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, were enacted in March 2013. It is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. Moreover, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates and companion diagnostic. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates and companion diagnostic. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the U.S. PTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The U.S. PTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

Issued patents covering our product candidates and companion diagnostic could be found invalid or unenforceable if challenged in court or the U.S. PTO.

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates or companion diagnostic, the defendant could counterclaim that the patent covering our product candidate or companion diagnostic, as applicable, is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous ground upon which a third party can assert invalidity or unenforceability of a patent Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates or related companion diagnostics. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates and companion diagnostic. Such a loss of patent protection could have a material adverse impact on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the U.S. PTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the recent case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the U.S. PTO may impact the value of our patents.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do

not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapies or small molecule compounds that are similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have exclusively licensed may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they could significantly harm our business, results of operations and prospects.

Risks Related to Ownership of our Common Stock

The market price of our common stock may be highly volatile, and you may not be able to resell some or all of your shares at a desired market price.

The market price of our common stock has been and is likely to continue to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- adverse results or delays in preclinical studies or clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;

- unanticipated serious safety concerns related to the use of any of our product candidates;
- reports of adverse events in other gene therapy products or clinical trials of such products;
- inability to obtain additional funding;
- any delay in filing an IND or BLA for any of our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND or BLA;
- failure to obtain regulatory and marketing approvals;
- sales or potential sales of our common stock by us or our stockholders in the future;
- failure to successfully develop, manufacturers, and commercialize our product candidates or companion diagnostic;
- failure to enter into collaborations;
- failure by us or our licensors to prosecute, maintain or enforce our intellectual property rights;
- our dependence on third parties, including, commercial manufactures, CROs as well as our partners that provide us with our companion diagnostic product;
- changes in laws or regulations applicable to future products;
- inability to obtain adequate clinical and commercial product supply for our product candidates or companion diagnostic or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- introduction of new products, services or technologies by our competitors;
- failure to meet or exceed financial projections we may provide to the public;
- failure to meet or exceed the financial projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;
- changes in the market valuations of similar companies;
- overall performance of the equity markets and other factors that may be unrelated to our operating performance or the operating performance of our competitors, including changes in market valuations of similar companies; and
- trading volume of our common stock.

In addition, companies trading in the stock market in general, and the NASDAQ Global Market in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

If securities or industry analysts issue an adverse or misleading opinion regarding our stock, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. If any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our clinical trials and operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Our executive officers, directors, 5% stockholders and their affiliates currently beneficially own a significant percentage of our outstanding voting stock. Therefore, these stockholders have the ability and may continue to have the ability to influence us through this ownership position. These stockholders may be able to determine some or all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

We are an "emerging growth company," and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies," including exemption from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 (Sarbanes-Oxley Act), reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of our initial public offering, (b) in which we have total annual gross revenue of at least $1.0 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeded $700.0 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expenses related to our product candidates, companion diagnostic or future development programs;
- if any of our product candidates receives regulatory approval, the level of underlying demand for these product candidates and wholesalers' buying patterns;
- addition or termination of clinical trials or funding support;

- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements.
- any intellectual property infringement lawsuit in which we may become involved; and
- regulatory developments affecting our product candidates or companion diagnostic or those of our competitors.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404 of the Sarbanes-Oxley Act, or the subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our consolidated financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales may have on the prevailing market price of our common stock.

Certain holders of our securities are entitled to rights with respect to the registration of their shares under the Securities Act of 1933, as amended, or the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by our affiliates as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2013 equity incentive plan, or the 2013 plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares available for future grant under the 2013 plan will automatically increase on January 1 of each year by 5% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, subject to the ability of our board of directors to take action to reduce the size of the increase in any given year. In addition, we may grant or provide for the grant of rights to purchase shares of our common stock pursuant to our 2013 employee stock purchase plan, or ESPP. The number of shares of our common stock reserved for issuance under the ESPP will automatically increase on January 1 of each calendar year by the lesser of 1% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year and 384,307 shares, subject to the ability of our board of directors to take action to reduce the size of the increase in any given year. Currently, we plan to register the increased number of shares available for issuance under the 2013 plan and ESPP each year. Increases in the number of shares available for future grant or purchase may result in additional dilution, which could cause our stock price to decline. In addition, we have in the past and may in the future grant inducement grants to prospective employees and consultants, which may result in further dilution and cause our stock price to decline.

We are at risk of securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biotechnology companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

Under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We have determined that several ownership changes have occurred since our inception and have reduced our deferred tax asset accordingly. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition our ability to pay dividends is currently restricted by the terms of our loan agreement with Hercules. Any return to stockholders will therefore be limited to the appreciation of their stock.

Provisions in our amended and restated certificate of incorporation and bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.

Some provisions of our charter documents and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders and may prevent attempts by our stockholders to replace or remove our current management. These provisions include:

- authorizing the issuance of "blank check" preferred stock, the terms of which may be established and shares of which may be issued without stockholder approval;
- limiting the removal of directors by the stockholders;

- creating a staggered board of directors;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- eliminating the ability of stockholders to call a special meeting of stockholders; and
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, which is responsible for appointing the members of our management. In addition, we are subject to Section 203 of the Delaware General Corporation Law, which generally prohibits a Delaware corporation from engaging in any of a broad range of business combinations with an interested stockholder for a period of three years following the date on which the stockholder became an interested stockholder, unless such transactions are approved by our board of directors. This provision could have the effect of delaying or preventing a change of control, whether or not it is desired by or beneficial to our stockholders. Further, other provisions of Delaware law may also discourage, delay or prevent someone from acquiring us or merging with us.

Our employment agreements with our executive officers and certain other employees may require us to pay severance benefits to any of those persons who are terminated under specified circumstances, including in connection with a change of control of us, which could harm our financial condition or results.

Our executive officers and certain other employees are parties to employment agreements that contain change of control and severance provisions providing for severance and other benefits and acceleration of vesting of stock options in the event of a termination of employment under specified circumstances, including in connection with a change of control of us. The accelerated vesting of options could result in dilution to our existing stockholders and harm the market price of our common stock. The payment of these severance benefits could harm our financial condition and results. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

ITEM 1B. Unresolved Staff Comments

Not applicable.

ITEM 2. Properties

Our corporate headquarters are located at 11988 El Camino Real, Suite 650, San Diego, California 92130 in a facility we lease encompassing approximately 10,908 square feet of office space. The lease for this facility expires in September 2021. We also have a short-term lease in Seattle, Washington for approximately 7,000 square feet of office space expiring in June 2016, and are exploring options for a longer term lease for office and laboratory space in Seattle.

ITEM 3. Legal Proceedings

We are not a party to any material legal proceedings at this time. From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

ITEM 4. Mine Safety Disclosures

Not applicable.

PART II

ITEM 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common stock began trading on The NASDAQ Global Market on January 30, 2014 and trades under the symbol "CLDN". Prior to January 30, 2014, there was no public market for our common stock. The following table sets forth the high and low sales prices per share of our common stock as reported on The NASDAQ Global Market for the period indicated.

	Price Range	
	High	Low
Year Ended December 31, 2014		
First Quarter (commencing January 30, 2014)	$17.16	$7.45
Second Quarter	$16.47	$7.82
Third Quarter	$16.72	$9.20
Fourth Quarter	$20.85	$9.20

Holders of Record

As of March 13, 2015, there were approximately 24 stockholders of record of our common stock. Certain shares are held in "street" name and accordingly, the number of beneficial owners of such shares is not known or included in the foregoing number.

Dividend Policy

We have never declared or paid any dividends on our common stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. In addition, our ability to pay dividends is currently restricted by the terms of our loan agreement with Hercules. Any future determination to pay dividends, if permitted, will be made at the discretion of our board of directors.

Recent Sales of Unregistered Securities

During the year ended December 31, 2014, we issued and sold the following unregistered securities:

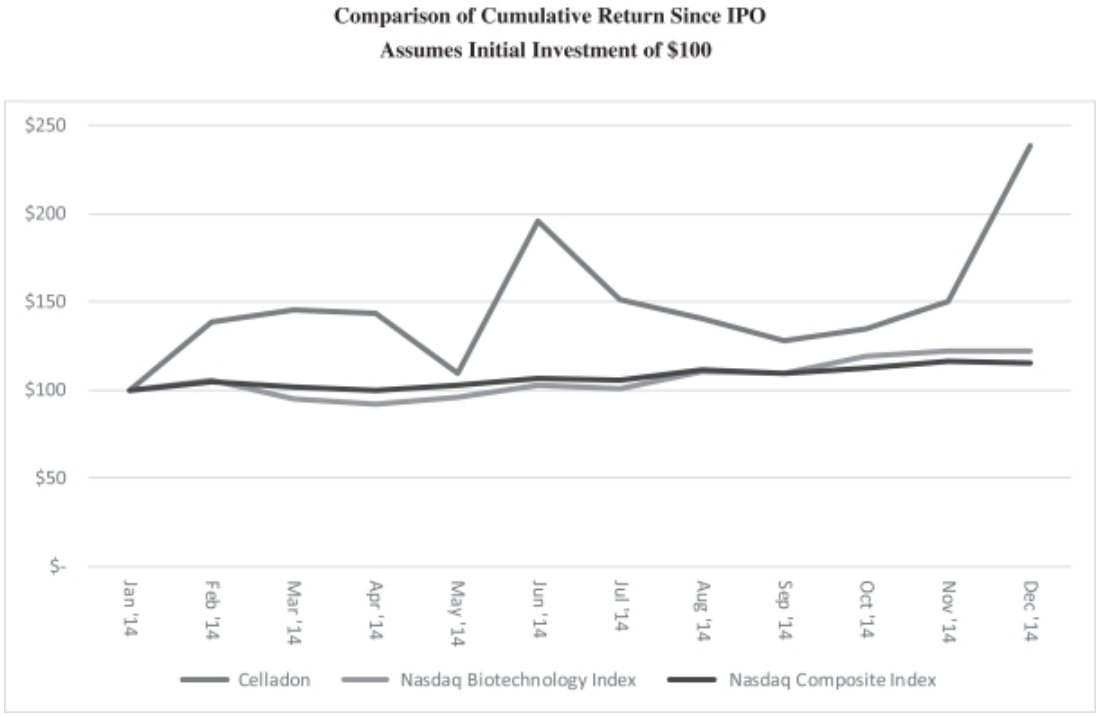
On May 27, 2014, we issued 25,481 shares of our common stock to Novartis International Pharmaceutical Investment Ltd pursuant to the exercise of a warrant for approximately $0.1 million in cash. The offer, sale and issuance of the foregoing shares of common stock were deemed to be exempt from registration under the Securities Act in reliance on Section 4(a)(2) of the Securities Act and Rule 506 promulgated under Regulation D thereunder as transactions by an issuer not involving a public offering.

Securities Authorized for Issuance Under Equity Compensation Plans

Information about our equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

Performance Graph

The following graph shows a comparison from January 30, 2014 (the date our common stock commenced trading on The NASDAQ Global Market) through December 31, 2014 of the cumulative total return for our common stock, the NASDAQ Biotechnology Index (NBI) and the NASDAQ Composite Index (CCMP). The graph assumes an initial investment of $100 on January 30, 2014. The comparisons in the graph are not intended to forecast or be indicative of possible future performance of our common stock.



Use of Proceeds

On January 29, 2014, the SEC declared effective the registration statement on Form S-1 (File Nos. 333-191688 and 333-193647) for our initial public offering of our common stock. Pursuant to the registration statement, we registered the offer and sale of 6,325,000 shares of our common stock. On February 4, 2014, we sold 5,500,000 shares of our common stock at a public offering price of $8.00 per share and on February 27, 2014, we sold 825,000 shares of our common stock at a public offering price of $8.00 per share pursuant to the full exercise of the underwriters' option to purchase additional shares. The offering has terminated. The sole book-running managing underwriter for the offering was Barclays Capital Inc. After deducting underwriting discounts, commissions and offering costs paid by us of $6.3 million, the net proceeds from the offering were approximately $44.3 million. No offering expenses were paid or are payable, directly or indirectly, to our directors or officers, to persons owning 10% or more of any class of our equity securities, or to any of our affiliates.

The net proceeds from our initial public offering have been invested in highly-liquid money market funds and investment grade corporate debt securities, pending their use. Our cash requirements for the year ended December 31, 2014 were primarily met with sources of liquidity available to us at December 31, 2013, prior to our initial public offering. As of December 31, 2014, we have used approximately $11.6 million of the net proceeds from our initial public offering. There has been no material change in the expected use of the net proceeds from our initial public offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b), except we intend to dedicate all or a portion of the net proceeds received by us from the full exercise of the underwriters' option to purchase additional shares to a trial of MYDICAR for the treatment of AVF maturation failure.

ITEM 6. Selected Financial Data

The following selected financial data should be read in conjunction with our audited financial statements located elsewhere in this Annual Report on Form 10-K and "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations". Amounts are in thousands, except share and per share data.

	Years Ended December 31,			Six Months Ended December 31,	Year Ended
	2014	2013	2012	2011	June 30, 2011
Consolidated Statements of Operations Data:					
Operating expenses:					
Research and development	$ 22,676	$ 16,927	$ 13,314	$ 1,252	$ 4,193
General and administrative	10,342	3,037	2,631	920	1,832
Total operating expenses	33,018	19,964	15,945	2,172	6,025
Loss from operations	(33,018)	(19,964)	(15,945)	(2,172)	(6,025)
Other income (expense)	(835)	(127)	74	(689)	(965)
Consolidated net loss	(33,853)	(20,091)	(15,871)	(2,861)	(6,990)
Net loss attributable to non-controlling interest	—	96	154	—	—
Net loss attributable to Celladon Corporation	(33,853)	(19,995)	(15,717)	(2,861)	(6,990)
Accretion to redemption value of redeemable convertible preferred stock	—	—	(343)	—	—
Change in fair value of non-controlling interest	—	(3,105)	(154)	—	—
Deemed dividend	—	(856)	—	—	—
Net loss attributable to common stockholders	$ (33,853)	$ (23,956)	$ (16,214)	$ (2,861)	$ (6,990)
Net loss per share attributable to common stockholders, basic and diluted (1)	$ (1.82)	$ (27.09)	$ (19.74)	$ (1,022.52)	$ (2,729.66)
Weighted-average shares outstanding, basic and diluted	18,603,605	884,179	821,568	2,798	2,561

	As of December 31,			
	2014	2013	2012	2011
Consolidated Balance Sheet Data:				
Cash, cash equivalents and investments	$ 84,948	$ 18,370	$ 35,511	$ 468
Working capital (deficit)	81,477	11,990	31,159	(14,835)
Total assets	89,110	21,154	35,929	636
Redeemable non-controlling interest	—	—	4,814	—
Redeemable convertible preferred stock	—	60,098	52,274	—
Convertible preferred stock	—	5,450	5,450	56,282
Deficit accumulated	(146,439)	(112,586)	(92,591)	(76,874)
Total stockholders' equity (deficit)	72,104	(50,991)	(28,416)	(70,979)

(1) See Note 1 to our consolidated financial statements appearing elsewhere in this Report for an explanation of the method used to calculate basic and diluted net loss per share attributable to common stockholders and the number of shares used in the computation of the per share amounts.

ITEM 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis together with "Item 6. Selected Financial Data" and our financial statements and related notes included elsewhere in this Annual Report. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those expressed or implied in any forward-looking statements as a result of various factors, including those set forth under the caption "Item 1A. Risk Factors."

Overview

We are a clinical-stage biotechnology company with industry-leading expertise in the development of cardiovascular gene therapy. We apply our leadership position in the field of gene therapy and calcium dysregulation to develop novel therapies for diseases with high unmet medical needs. Our therapeutic portfolio for diseases characterized by SERCA enzyme deficiency includes both gene therapies and small molecule compounds. We are evaluating our lead product candidate, MYDICAR, in a 250-patient randomized, double-blind, placebo-controlled international Phase 2b trial in patients with heart failure for reduced ejection fraction or HFrEF (also referred to as systolic heart failure), which we refer to as CUPID 2. We completed enrollment of CUPID 2 in February 2014, reached the primary analysis data cutoff in February 2015 and expect to unblind the data and announce results in late April 2015. If successful, these results, along with other studies, could form the basis for regulatory submissions for approval with the United States Food and Drug Administration, or FDA, and European Medicines Agency, or EMA. We also plan to develop MYDICAR for additional indications, such as AVF maturation failure, and for the treatment of patients with advanced heart failure who are on an LVAD. Subject to raising additional capital, we also may initiate development programs in heart failure for preserved ejection fraction or HFpEF (also referred to as diastolic heart failure), which is caused by the inability of the heart to relax normally between contractions, and pulmonary arterial hypertension, or PAH, which is characterized by a SERCA2a deficiency in VSMC. MYDICAR has demonstrated potential disease-modifying capability in preclinical models of these diseases. We currently hold worldwide rights to MYDICAR and plan to commercialize MYDICAR for all approved heart failure indications using a targeted sales force in the United States focused on selected cardiologists and heart failure specialists who treat the majority of heart failure patients.

We have incurred net losses in each year since our inception. Our consolidated net losses were approximately $33.9 million and $20.1 million for the years ended December 31, 2014 and 2013, respectively. As of December 31, 2014, we had an accumulated deficit of approximately $146.4 million. Substantially all our net losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We anticipate that our expenses will increase substantially if and as we:

- further validate and develop the manufacturing process for MYDICAR and our companion diagnostic, including commercial scale-up and contract for the construction and operation of one or more commercial manufacturing facilities, and validate and develop manufacturing processes for our other product candidates and any related companion diagnostics;
- initiate, expand or accelerate preclinical and clinical development activities for our lead product candidate, MYDICAR, including with respect to clinical trials of MYDICAR for HFrEF. Ongoing and planned clinical trials of MYDICAR for HFrEF include CUPID 2, CUPID 3, the LVAD trial, the AAV1 NAb positive trial, the viral shedding trial and a higher dose trial. If supported by data, we also plan to conduct preclinical and potentially clinical activities to evaluate MYDICAR for the treatment of AVF maturation failure, and clinical trials of MYDICAR for the treatment of heart failure for preserved ejection fraction, or HFpEF, (also referred to as diastolic heart failure) and other indications such as PAH;
- advance our additional preclinical assets, including our stem cell factor gene therapy and our small molecule platform targeting SERCA2 enzymes;

- continue our research and preclinical development of our product candidates and seek to identify and validate additional product candidates;
- seek regulatory and marketing approvals for MYDICAR and its companion diagnostic and any other product candidate that successfully completes clinical trials;
- establish a sales, marketing and distribution infrastructure in the United States to commercialize any products for which we obtain marketing approval;
- acquire rights to other product candidates and technologies;
- change or add manufacturers or suppliers;
- maintain, expand and protect our intellectual property portfolio;
- make milestone or other payments under any in-license or collaboration agreement;
- attract and retain skilled personnel;
- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with any of the above.

We expect to continue to incur significant expenses and increasing losses for at least the next several years. Accordingly, we anticipate that we will need to raise additional capital prior to the commercialization of MYDICAR, our companion diagnostic, our small molecule program, or any of our other product candidates. Until such time that we can generate meaningful revenue from product sales, if ever, we expect to finance our operating activities through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates and companion diagnostic. If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any approved products or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially adversely affect our business, financial condition and results of operations.

Financial Overview

Research and Development Expenses

To date, we have devoted substantially all of our resources to research and development efforts relating to our product candidates, including conducting clinical trials, developing manufacturing capabilities, in-licensing related intellectual property, providing general and administrative support for these operations and protecting our intellectual property. We recognize research and development expenses as they are incurred. Our research and development expenses consist primarily of:

- salaries and related overhead expenses, which include stock-based compensation and benefits for personnel in research and development functions;
- fees paid to contract manufacturers for commercial scale-up activities;
- fees paid to consultants and contract research organizations, or CROs, including in connection with our preclinical studies and clinical trials and other related clinical trial fees, such as for investigator grants, patient screening, laboratory work, clinical trial material management and statistical compilation and analysis;
- costs related to acquiring and manufacturing clinical trial materials, including continued testing such as process validation and stability of drug product;

- costs related to compliance with regulatory requirements; and
- payments related to licensed products and technologies.

From our inception through December 31, 2014, we have incurred approximately $114.7 million in research and development expenses, of which we estimate $108.7 million relates to our development of MYDICAR. We plan to increase our research and development expenses for the foreseeable future as we continue to develop MYDICAR for the treatment of HFrEF and our companion diagnostic, as well as, subject to the availability of additional funding, further advance the development of our other product candidates and MYDICAR for additional indications. Our direct research and development expenses consist principally of external costs, such as fees paid to investigators, consultants, central laboratories and CROs, in connection with our clinical trials, developing manufacturing capabilities and costs related to acquiring and manufacturing clinical trial materials. We typically use our employee and infrastructure resources across multiple research and development programs.

The successful development of our clinical and preclinical product candidates and companion diagnostic is highly uncertain. At this time, we cannot reasonably estimate the nature, timing or costs of the efforts that will be necessary to complete the remainder of the development of any of our clinical or preclinical product candidates or companion diagnostic or the period, if any, in which material net cash inflows from these product candidates may commence. This is due to the numerous risks and uncertainties associated with the development of our product candidates and companion diagnostic, including:

- the uncertainty of the scope, rate of progress and expense of our ongoing, as well as additional, clinical trials and other research and development activities;
- the potential benefits of our product candidates over other therapies;
- our ability to market, commercialize and achieve market acceptance for any product candidate or companion diagnostic that we are developing or may develop in the future;
- ongoing and future clinical trial results;
- the timing and receipt of any regulatory approvals of MYDICAR for HFrEF, and approval to initiate a clinical trial to evaluate MYDICAR for the treatment of AVF maturation failure, an AAV1 NAb positive trial and a viral shedding trial; and
- the filing, prosecuting, defending and enforcing of patent claims and other intellectual property rights, and the expense of doing so.

A change in the outcome of any of these variables with respect to the development of a product candidate or companion diagnostic could mean a significant change in the costs and timing associated with the development of that product candidate or companion diagnostic. For example, if the FDA, the EMA or other foreign regulatory authorities were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate or companion diagnostic, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time with respect to the development of that product candidate or companion diagnostic.

MYDICAR

The majority of our research and development resources are currently focused on our ongoing CUPID 2 trial, commercialization and manufacturing preparations, clinical trials and other work needed to submit MYDICAR for regulatory approval in the United States and Europe. We have incurred, and expect to continue to incur, significant expense in connection with these efforts, including expenses related to:

- the development of manufacturing capabilities for the commercial production of MYDICAR;

- conduct of our CUPID 2 trial of MYDICAR and the enrollment and conduct of an AVF trial, AAV NAb positive trial, viral shedding trial for patients with HFrEF, exploring the feasibility of plasma exchange in removing AAV1 NAbs in advanced heart failure patients prior to treatment with MYDICAR, and research and, pending outcome of CUPID2 data, clinical development of MYDICAR for the treatment of HFpEF; and
- commercial scale-up, validation and automation activities related to our companion diagnostic.

Small Molecule Program

Our research and development expenses for our small molecule program relate primarily to identification and pre-clinical testing of small molecule SERCA2 enzyme modulators.

Stem Cell Factor Program

Our research and development expenses for our stem cell factor program relate primarily to the identification of potential gene therapy applications of the membrane-bound form of the Stem Cell Factor gene (mSCF).

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for employees in executive, legal, finance and administration, corporate development and administrative support functions, including stock-based compensation expenses and benefits. Other significant general and administrative expenses include accounting and legal services, expenses associated with obtaining and maintaining patents, the cost of various consultants, occupancy costs and information systems costs.

Other Income (Expense)

Other expense consists primarily of the accretion of debt discount and interest charges on our current and prior debt agreements and the change in the fair value of our outstanding warrant liability prior to its reclassification to stockholders' equity in February 2014 in connection with the closing of our initial public offering. Other income consists primarily of interest income earned on our cash, cash equivalents and investments.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of our consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our consolidated financial statements, as well as the reported expenses during the reported periods. We evaluate these estimates and judgments on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 1 to our consolidated financial statements appearing elsewhere in this Annual Report, we believe that the following accounting policies related to clinical trial expenses and valuation of stock-based compensation are the most critical for fully understanding and evaluating our financial condition and results of operations.

Clinical Trial Accruals

As part of the process of preparing our consolidated financial statements, we are required to estimate our expenses resulting from our obligations under contracts with vendors and consultants and clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations which vary from contract to contract and may result in payment flows that do not match the periods over which materials or services are provided to us under such contracts. Our clinical trial accrual is dependent upon the timely and accurate reporting of CROs and other third-party vendors.

Our objective is to reflect the appropriate clinical trial expenses in our consolidated financial statements by matching those expenses with the period in which services and efforts are expended. We account for these expenses according to the progress of the trial as measured by patient progression and the timing of various aspects of the trial. We determine accrual estimates through discussion with applicable personnel and outside service providers as to the progress or state of completion of clinical trials, or the services completed. During the course of a clinical trial, we adjust the rate of clinical trial expense recognition if actual results differ from the estimates. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known at that time. Although we do not expect that our estimates will be materially different from amounts actually incurred, our understanding of status and timing of services performed relative to the actual status and timing of services performed may vary and may result in our reporting amounts that are too high or too low for any particular period. Through December 31, 2014, there had been no material adjustments to our prior period estimates of accrued expenses for clinical trials. However, due to the nature of estimates, we cannot assure you that we will not make changes to our estimates in the future as we become aware of additional information about the status or conduct of our clinical trials.

Stock-Based Compensation

Compensation expense for stock-based payment awards made to our employees and directors, including employee stock options and employee stock purchases related to the Employee Stock Purchase Plan (ESPP), represents the grant date fair value of the awards recognized over the requisite service period (usually the vesting period) on a straight-line basis, net of estimated forfeitures. For stock option grants with performance-based milestones, the expense is recorded over the remaining service period after the point when the achievement of the milestone is probable or the performance condition has been achieved.

We account for stock options granted to non-employees using the fair-value approach. These options are subject to periodic revaluation over their vesting terms.

We estimate the fair value of our stock-based payment awards using the Black-Scholes option pricing model, which requires the input of highly subjective assumptions, including (a) the risk-free interest rate, (b) the expected volatility of our stock, (c) the expected term of the award and (d) the expected dividend yield. Until our recently completed initial public offering, there was no public market for the trading of our common stock. Due to this fact and a lack of company specific historical and implied volatility data, we have based our estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. For these analyses, we have selected companies with comparable characteristics to ours, including enterprise value, risk profiles, position within the industry and with historical share price information sufficient to meet the expected life of the stock-based awards. We compute the historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of our stock-based awards. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available. We have estimated the expected life of our employee stock options using the "simplified" method, whereby, the expected life equals the average of the vesting term and the original contractual term of the option. The risk-free interest rate is based on U.S. Treasury zero-coupon bonds with maturities similar to those of the expected term of the award being valued.

Other Information

Net Operating Loss Carryforwards

As of December 31, 2014, we had federal and California tax net operating loss carryforwards of approximately $82.2 million and $77.0 million, respectively. The federal net operating loss carryforwards will begin to expire in 2027 unless previously utilized, and the state net operating loss carryforwards have already begun to expire, and will continue to do so, unless utilized. As of December 31, 2014, we had federal and California research and development tax credit carryforwards of approximately $1.3 million each. The federal research and development tax credit carryforwards will begin to expire in 2032, unless previously utilized. The California research and development tax credit carryforwards are available indefinitely until utilized.

The future utilization of net operating loss and tax credit carryforwards may be limited due to changes in ownership. In general, if we experience a greater than 50% aggregate change in ownership of certain significant stockholders or groups over a three-year period, or a Section 382 ownership change, utilization of our pre-change net operating loss carryforwards would be subject to an annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, and similar state laws. The annual limitation is generally determined by multiplying the value of our stock at the time of such ownership change (subject to certain adjustments) by the applicable long-term tax-exempt rate. Such limitations may result in expiration of a portion of the pre-change net operating loss carryforwards before utilization and may be substantial. We completed a study to assess whether an ownership change, as defined by Section 382 of the Code, had occurred since our formation through December 31, 2014. Based upon the results of this study, we determined that several ownership changes had occurred and we reduced our deferred tax asset with a corresponding adjustment to the valuation allowance accordingly. We have recorded a valuation allowance for the full amount of the remaining portion of the deferred tax asset related to our net operating loss and research and development tax credit carryforwards. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us.

JOBS Act

On April 5, 2012, the JOBS Act was enacted. Section 107 of the JOBS Act provides that an “emerging growth company” can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. In other words, an “emerging growth company” can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

Subject to certain conditions set forth in the JOBS Act, as an “emerging growth company,” we intend to rely on certain of these exemptions, including without limitation, (1) providing an auditor’s attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (2) complying with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the consolidated financial statements, known as the auditor discussion and analysis. We will remain an “emerging growth company” until the earliest of (a) the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more, (b) the last day of our fiscal year following the fifth anniversary of the date of the completion of our initial public offering, (c) the date on which we have issued more than $1 billion in non-convertible debt during the previous three years or (d) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Results of Operations

Comparison of the Years Ended December 31, 2014 and 2013

The following table summarizes our results of operations for the years ended December 31, 2014 and 2013 (in thousands):

	Years Ended December 31,		Increase / (Decrease)
	2014	2013	
Research and development	$22,676	$16,927	$ 5,749
General and administrative	10,342	3,037	7,305
Total other income (expense)	(835)	(127)	(708)

Research and Development Expenses. Research and development expenses were $22.7 million and $16.9 million for the years ended December 31, 2014 and 2013, respectively. The increase of approximately $5.7 million was due primarily to an increase of $5.7 million in expenses during 2014 associated with the drug substance manufacturing scale-up, $1.7 million in personnel costs related to an increase in headcount, $0.6 million in non-clinical studies related to MYDICAR, $0.4 million in stock-based compensation, $0.3 million in consulting, regulatory and other costs offset by a decrease of $3.0 million in clinical costs due to the completion of enrollment in our CUPID2 trial in the first quarter of 2014. We expect that our overall research and development expenses will increase in 2015 as we initiate additional clinical trials and continue manufacturing scale-up activities.

General and Administrative Expenses. General and administrative expenses were $10.3 million and $3.0 million for the years ended December 31, 2014 and 2013, respectively. The increase of approximately $7.3 million was due primarily to an increase of $2.5 million in compensation expense related to an increase in headcount, $2.1 million in costs associated with operating as a publicly traded company, including investor relations, legal, audit, insurance, taxes and director fees, $1.5 million in stock-based compensation, $0.7 million in marketing costs and $0.5 million in patent, office and other costs. We expect that our general and administrative expenses will increase as we continue to operate as a public company, including costs to comply with corporate governance and internal controls, and as we add personnel to support product commercialization efforts.

Other Expense. Other expense was $0.8 million and $0.1 million for the years ended December 31, 2014 and 2013, respectively. The other expense for the year ended December 31, 2014 consisted primarily of $0.7 million of expense related to the accretion of debt discount and interest charges on our term loan, $0.2 million increase in fair value of the warrant liability prior to reclassification to equity upon our initial public offering and $29,000 foreign currency exchange loss offset by $0.1 million in interest income on our investments. The other expense for the year ended December 31, 2013 consisted primarily of $0.2 million of other expense related to an increase in the fair value of the outstanding warrant liability and $45,000 of interest expense related to the amortization of debt discount on the outstanding convertible debt, offset by $0.1 million of interest income on our investments and $25,000 foreign currency exchange gain.

Comparison of the Years Ended December 31, 2013 and 2012

The following table summarizes our results of operations for the years ended December 31, 2013 and 2012 (in thousands):

	Years Ended December 31,		Increase / (Decrease)
	2013	2012	
Research and development	$16,927	$13,314	$ 3,613
General and administrative	3,037	2,631	406
Total other income (expense)	(127)	74	(201)

Research and Development Expenses. Research and development expenses were $16.9 million and $13.3 million for the years ended December 31, 2013 and 2012, respectively. The increase of approximately $3.6 million was due primarily to an increase of $4.5 million in expenses during 2013 associated with the increase in enrollment of patients in our CUPID 2 clinical trial, $0.8 million associated with the transfer of our manufacturing process to Lonza and $1.5 million in compensation related to an increase in headcount and stock-based compensation, offset by a charge of $3.2 million which occurred during the year ended December 31, 2012 related to the purchase of intangible assets from AmpliPhi Biosciences Corporation, or AmpliPhi, relating to the development of MYDICAR.

General and Administrative Expenses. General and administrative expenses were $3.0 million and $2.6 million for the years ended December 31, 2013 and 2012, respectively. The increase of approximately $0.4 million was due primarily to an increase in compensation expense related to an increase in headcount and professional fees associated with transitioning our company into a public company, offset by a reduction in outside legal services due to the completion of the establishment of our former European subsidiary, Celladon Europe, and legal costs associated with licensing activities in 2012.

Other Income (Expense). Other income (expense) was $(0.1) million and $74,000 for the years ended December 31, 2013 and 2012, respectively. The other expense for the year ended December 31, 2013 consisted primarily of $0.2 million of other expense related to an increase in the fair value of our outstanding warrant liability and $45,000 of interest expense related to the amortization of debt discount on our outstanding convertible debt, offset by $0.1 million of interest income on our investments and $25,000 foreign currency exchange gain. The other income for the year ended December 31, 2012 consisted primarily of interest income on our investments offset by interest expense on outstanding convertible debt.

Liquidity and Capital Resources

We have incurred net losses each year since our inception and as of December 31, 2014, we had an accumulated deficit of approximately $146.4 million. We anticipate that we will continue to incur net losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may obtain through one or more public or private equity offerings, debt financings, government or other third-party funding, strategic alliances and licensing or collaboration arrangements.

Since our inception through December 31, 2014, we have funded our operations primarily from the sale of our equity and debt securities. As of December 31, 2014, we had cash, cash equivalents and investments of approximately $84.9 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Our initial public offering completed in February 2014 resulted in net proceeds to us of $44.3 million after deducting underwriting discounts and commission and offering expenses payable by us, including $1.7 million in offering costs paid by us prior to December 31, 2013. The outstanding principal and accrued interest thereon under our outstanding convertible promissory notes converted into shares of our common stock upon the closing of our initial public offering. On July 31, 2014, we entered into a Loan Agreement with Hercules under which we can borrow up to $25.0 million in two tranches. On August 1, 2014, we borrowed the first tranche in the amount of $10.0 million and received $9.6 million, net of fees. The second tranche of up to $15.0 million can be drawn at our option through June 30, 2015 (amended from May 31, 2015), subject to the satisfaction of certain funding conditions related to our clinical development of MYDICAR. Our underwritten public offering completed in August 2014 resulted in net proceeds to us of $40.7 million after deducting underwriting discounts and commission and offering expenses payable by us.

The following table shows a summary of our cash flows for the periods indicated (in thousands):

	Years Ended December 31,		
	2014	2013	2012
Net cash provided by (used in):			
Operating activities	$(29,259)	$(16,196)	$(14,637)
Investing activities	(61,188)	10,854	(21,833)
Financing activities	96,979	(596)	49,843
Net (decrease) increase in cash and cash equivalents	$ 6,532	$ (5,938)	$ 13,373

Operating activities. Net cash used in operating activities of $29.3 million during the year ended December 31, 2014 was primarily a result of our net loss of $33.9 million. The primary difference between our net loss and our cash used in operating activities was $3.3 million of non-cash stock-based compensation, $0.4 million of interest income related to the amortization of discounts and premiums paid on investment securities, $0.4 million of noncash interest related to the accretion of debt discount on our term loan, $0.2 million related to the change in fair value of our outstanding warrant liability, $0.2 million of depreciation expense and $0.1 million relating to changes in our operating assets and liabilities.

Net cash used in operating activities of $16.2 million during the year ended December 31, 2013 was primarily a result of our net loss of $20.1 million. The primary difference between our net loss and our cash used in operating activities was $1.9 million of changes in our operating assets and liabilities, $1.4 million of stock-based compensation, $0.2 million related to the change in fair value of our outstanding warrant liability, $0.3 million of interest income related to the amortization of discounts and premiums paid on investment securities and $0.1 million of noncash interest related to the amortization of debt discount on our convertible debt.

Net cash used in operating activities of $14.6 million during the year ended December 31, 2012 was primarily a result of our net loss of $15.9 million. The primary difference between our net loss and our cash used in operating activities was $0.6 million of changes in our operating assets and liabilities and $0.3 million of stock-based compensation.

Investing Activities. Net cash used in investing activities of $61.2 million during the year ended December 31, 2014 was primarily a result of $60.5 million in net purchases of investment securities and $0.7 million in purchases of property and equipment.

Net cash provided by investing activities of $10.9 million during the year ended December 31, 2013 was primarily a result of the net maturities of investments used to fund our operating activities.

Net cash used in investing activities was $21.8 million during the year ended December 31, 2012 and consisted primarily of the investment of proceeds received from our Series A-1 preferred stock financing.

Financing Activities. Net cash provided by financing activities during the year ended December 31, 2014 consisted primarily of $94.3 million in proceeds received and $7.7 million in costs paid in connection with our public offerings, $9.6 million in net borrowings under our term loan, $0.7 million in proceeds from the exercise of stock options and sale of shares under our employee stock purchase plan and $0.1 million in proceeds upon the exercise of warrants in exchange for common stock.

Net cash used in financing activities during the year ended December 31, 2013 consisted of $1.7 million of costs we paid in connection with our IPO, offset by $1.1 million of proceeds from our issuance of convertible debt.

Net cash provided by financing activities of $49.8 million during the year ended December 31, 2012 was primarily a result of proceeds received from our Series A-1 preferred stock financing and related issuance of exchangeable shares.

Future Funding Requirements

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate any revenue from product sales unless and until we obtain regulatory approval of MYDICAR and our companion diagnostic and commercialize MYDICAR or any of our other product candidates. At the same time, we expect our expenses to increase in connection with our ongoing development activities, particularly as we continue the research, development and clinical trials of, and seek regulatory approval for, our product candidates and companion diagnostic. We expect to incur additional costs associated with operating as a public company. In addition, subject to obtaining regulatory approval of any of our product candidates and companion diagnostic, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. We anticipate that we will need additional funding in connection with our continuing operations.

Based upon our current operating plan, we believe that our existing cash, cash equivalents and short-term investments, as well as borrowings available to us under our loan facility with Hercules, will enable us to fund our operations for at least the next 12 months. We intend to use our existing cash, cash equivalents and short-term investments to fund development activities, including commercial manufacturing capabilities, related to MYDICAR for the treatment of HFrEF, including patients with existing LVADs, AVF maturation failure, internal salaries and external costs related to completion of our CUPID 2 trial and the remainder to fund working capital, including general operating expenses. We have based our estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the development of our product candidates.

Our future capital requirements will depend on many factors, including:

- the progress, costs, results and timing of our CUPID 2 trial, and the clinical development of MYDICAR for other potential indications;
- the willingness of the FDA to accept CUPID 2, as well as our other completed and planned preclinical studies and clinical trials and other work, as the basis for review and regulatory approval of MYDICAR for the treatment of HFrEF and for other potential indications;
- the outcome, costs and timing of seeking and obtaining FDA and any other regulatory approvals;
- the costs associated with securing, establishing and maintaining commercialization and manufacturing capabilities;
- the number and characteristics of product candidates that we pursue, including our product candidates in preclinical development;
- the ability of our product candidates to progress through clinical development successfully;
- our need to expand our research and development activities;
- the costs of acquiring, licensing or investing in businesses, products, product candidates and technologies;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;

- our need and ability to hire additional management and scientific, medical and sales personnel;
- the effect of competing technological and market developments; and
- our need to implement additional internal systems and infrastructure, including financial and reporting systems.

Until such time that we can generate meaningful revenue from product sales, if ever, we expect to finance our operating activities through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements, and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our common stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through government or other third-party funding, marketing and distribution arrangements or other collaborations, or strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at December 31, 2014 (in thousands):

	Payments due by period				
	Total	Less than 1 year	1 – 3 Years	3 – 5 Years	More than 5 years
Long-term obligations (1)	$11,762	$ 1	$7,485	$4,276	$ —
Interest commitment on long-term obligations (1)	1,980	840	1,107	33	—
Operating leases obligations	3,054	466	972	840	776
Total	$16,796	$1,307	$9,564	$5,149	$ 776

(1) Primarily consists of $10.0 million term loan borrowed by us on August 1, 2014 under our loan and security agreement with Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc. dated July 31, 2014. A final payment equal to $1.8 million will be due at such time as all amounts borrowed under the loan and security agreement are prepaid or become due and payable. The term loan has a scheduled maturity date of February 1, 2018. Also included in our long-term obligations is a nominal obligation for the capital lease of office equipment.

Additionally, we have entered and will continue to enter into contracts in the normal course of business with CROs for clinical trials and with vendors for preclinical research studies and other services and products for operating purposes. These agreements generally provide for termination or cancellation within 180 days or less of notice, and therefore are not included in the table above. We have also entered into an agreement with a contract manufacturer to conduct the initial work necessary to prepare for the potential manufacture of MYDICAR drug substance denominated in euros with a minimum contractual payments clause. As of December 31, 2014, we have a remaining obligation of €3.8 million which, under the terms of the contract, is expected to be paid within a one year period.

Each of our license agreements under which we may be required to pay an annual fee to maintain the license is generally cancelable by us, given appropriate prior written notice and, as such, is excluded from the table above. The annual amounts payable by us to maintain our existing licenses is approximately $0.6 million. In addition, we have payment obligations under license agreements that are contingent upon future events such as

our achievement of specified development, regulatory and commercial milestones and are required to make development milestone payments and royalty payments in connection with the sale of products developed under these agreements. As of December 31, 2014, we were unable to estimate the timing or likelihood of achieving the milestones or making future product sales and, therefore, any related payments are not included in the table above. We have potential milestone payment obligations of approximately $5.0 million, assuming only one product is developed or commercialized under each of our existing license agreements.

Off-Balance Sheet Arrangements

During the periods presented we did not have, nor do we currently have, any off-balance sheet arrangements as defined under the rules of the Securities and Exchange Commission.

Recent Accounting Pronouncements

In July 2013, the FASB issued Accounting Standards Update (ASU) No. 2013-11, *Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists*. ASU 2013-11 provides explicit guidance on the financial statement presentation of an unrecognized tax benefit when a net operating loss carryforward, a similar tax loss, or a tax credit carryforward exists. The guidance is effective prospectively for fiscal years, and interim periods within those years, beginning after December 15, 2013, with an option for early adoption. On January 1, 2014, we adopted this standard, which had no impact on our financial position or results of operations.

In June 2014, the FASB issued Accounting Standards Update (ASU) No. 2014-10, *Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*. ASU 2014-10 a) eliminates the requirement for development stage entities to present inception-to-date information in the statements of income, cash flows and shareholder equity, b) amends Topic 275 to clarify that the risk and uncertainty disclosure requirements apply to entities that have not commenced principal operations, c) eliminates the exception related to the sufficiency of equity at risk for development stage entities from the guidance on variable interest entities in paragraph 810-10-15-16 to increase consistency in application of consolidated guidance across all entities and d) removes the definition of *development stage entities* from the Master Glossary of the Accounting Standards Codification. The amendments in this Update are to be applied retrospectively except for the clarification to Topic 275, which shall be applied prospectively. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2014, with an option for early adoption. We adopted this guidance prior to filing this Annual Report on Form 10-K for the year ended December 31, 2014. The adoption of ASU 2014-10 impacted disclosure only and did not have any impact on financial position or results of operations.

In June 2014, the FASB issued ASU No. 2014-12, *Accounting for Share-Based Payments When the Terms of an Award Provide That a Performance Target Could Be Achieved after the Requisite Service Period*, which clarifies that entities should treat performance targets that can be met after the requisite service period of a share-based payment award as performance conditions that affect vesting. This standard is effective for annual reporting periods ending after December 15, 2015 and interim periods within those annual periods. Early application is permitted. The adoption of this guidance is expected to have no impact on our financial position or results of operations.

In August 2014, the FASB issued ASU No. 2014-15, *Presentation of Financial Statements Going Concern*, which requires management to assess an entity's ability to continue as a going concern, and to provide related footnote disclosure in certain circumstances. This standard is effective for annual reporting periods ending after December 15, 2016 and interim periods thereafter. Early application is permitted. The adoption of this guidance is expected to have no impact on our financial position or results of operations.

In November 2014, the FASB issued ASU No. 2014-16, Determining Whether the Host Contract in a Hybrid Financial Instrument Issued in the Form of a Share is More Akin to Debt or Equity, which requires the use of the whole instrument approach in determining the nature of a host contract in a hybrid instrument. This standard is effective for annual reporting periods ending after December 15, 2015 and interim periods within those annual periods. Early application is permitted. The adoption of this guidance is expected to have no impact on our financial position or results of operations.

ITEM 7A. Quantitative and Qualitative Disclosures About Market Risk

We have market risk exposure related to our cash, cash equivalents and investments. We invest our excess cash in highly liquid short-term investments such as money market funds. Changes in interest rates affect the investment income we earn on our investments and therefore impacts our cash flows and results of operations.

We do not believe that our cash, cash equivalents and investments have significant risk of default or illiquidity. While we believe our cash and cash equivalents do not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash and cash equivalents at one or more financial institutions that are in excess of federally insured limits.

We also have interest rate exposure as a result of our secured term loan with Hercules. As of December 31, 2014, the outstanding principal amount of the term loan was $10.0 million. The outstanding principal under the loan accrues interest at a rate equal to the greater of (i) 8.25% plus the prime rate as reported from time to time in The Wall Street Journal minus 5.25%, and (ii) 8.25%. Changes in the prime rate may therefore affect our interest expense associated with our secured term loan.

If a 10% change in interest rates from the interest rates on December 31, 2014 were to have occurred, this change would not have had a material effect on the value of our short-term investment portfolio or on our interest expense obligations with respect to outstanding borrowed amounts.

As of December 31, 2014, we had contractual payment obligations denominated in euros of approximately €3.8 million pursuant to our letter agreement with Novasep. We do not participate in any foreign currency hedging activities and we do not have any other derivative financial instruments. We did not recognize any significant exchange rate losses during the year ended December 31, 2014. A 10% change in the euro-to-dollar exchange rate on December 31, 2014 would not have had a material effect on our results of operations or financial condition.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation has had a material effect on our results of operations during the periods presented.

ITEM 8. Financial Statements and Supplementary Data

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of
Celladon Corporation

We have audited the accompanying consolidated balance sheets of Celladon Corporation, as of December 31, 2014 and 2013, and the related consolidated statements of operations and comprehensive income (loss), stockholders' (deficit) equity, and cash flows for each of the three years in the period ended December 31, 2014. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Celladon Corporation at December 31, 2014 and 2013, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2014, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

San Diego, California
March 31, 2015

Celladon Corporation

Consolidated Balance Sheets
(in thousands, except share and per share data)

	December 31	
	2014	2013
Assets		
Current assets:		
Cash and cash equivalents	$ 14,435	$ 7,903
Short-term investments	70,513	10,467
Prepaid expenses and other assets	3,135	180
Total current assets	88,083	18,550
Property and equipment, net	763	308
Other assets	264	2,296
Total assets	$ 89,110	$ 21,154
Liabilities, preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable and accrued expenses	$ 5,803	$ 2,908
Accrued clinical expenses	731	1,478
Accrued interest	71	14
Current portion of long-term obligations	1	—
Convertible notes, net of discount	—	1,044
Warrant liability	—	1,116
Total current liabilities	6,606	6,560
Term loan, net of discount	10,102	—
Non-current liabilities	298	37
Commitments and contingencies (Note 5)		
Series A-1 redeemable convertible preferred stock, $0.0001 par value:		
Authorized shares—none and 135,826,497 at December 31, 2014 and 2013, respectively; issued and outstanding shares—none and 127,140,530 at December 31, 2014 and 2013, respectively; liquidation preference—none and $114,172 at December 31, 2014 and 2013, respectively	—	60,098
Convertible preferred stock, $0.0001 par value:		
Authorized shares—none and 12,138,080 at December 31, 2014 and 2013, respectively; issued and outstanding shares—none and 12,138,080 at December 31, 2014 and 2013, respectively; liquidation preference—none and $5,450 at December 31, 2014 and 2013, respectively	—	5,450
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value; authorized shares—10,000,000 and none at December 31, 2014 and 2013, respectively; no shares issued and outstanding	—	—
Common stock, $0.001 par value; authorized shares—200,000,000 and 180,000,000 at December 31, 2014 and 2013, respectively; issued and outstanding—23,490,737 and 884,179 at December 31, 2014 and 2013, respectively	23	—
Additional paid-in capital	218,528	61,593
Accumulated other comprehensive (loss) income	(8)	2
Accumulated deficit	(146,439)	(112,586)
Total stockholders' equity (deficit)	72,104	(50,991)
Total liabilities, preferred stock and stockholders' equity (deficit)	$ 89,110	$ 21,154

See accompanying notes.

Celladon Corporation

Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)

	Years Ended December 31,		
	2014	2013	2012
Operating expenses:			
Research and development	$ 22,676	$ 16,927	$ 13,314
General and administrative	10,342	3,037	2,631
Total operating expenses	33,018	19,964	15,945
Loss from operations	(33,018)	(19,964)	(15,945)
Other income (expense):			
Interest income	118	69	35
Interest expense	(741)	(59)	(108)
Other (expense) income	(29)	25	147
Change in fair value of warrant liability	(183)	(162)	—
Consolidated net loss	(33,853)	(20,091)	(15,871)
Net loss attributable to non-controlling interest	—	96	154
Net loss attributable to Celladon Corporation	(33,853)	(19,995)	(15,717)
Accretion to redemption value of redeemable convertible preferred stock	—	—	(343)
Change in fair value of non-controlling interest	—	(3,105)	(154)
Deemed dividend	—	(856)	—
Net loss attributable to common stockholders	$ (33,853)	$ (23,956)	$ (16,214)
Other comprehensive loss:			
Unrealized (loss) gain on investments	(10)	(7)	9
Comprehensive loss	$ (33,863)	$ (20,098)	$ (15,862)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.82)	$ (27.09)	$ (19.74)
Weighted-average shares outstanding, basic and diluted	18,603,605	884,179	821,568

See accompanying notes.

Celladon Corporation

Consolidated Statements of Preferred Stock and Stockholders' Deficit
(in thousands, except share data)

	Series A-1 Redeemable Convertible Preferred Stock		Convertible Preferred Stock		Special Voting Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2011	—	$ —	39,186,807	$ 56,282	—	$ —	2,798	$ 5,895	$ —	$ —	$ (76,874)	$ (70,979)
Issuance of common stock	—	—	—	—	—	—	55	—	—	—	—	—
Conversion of Series A, B, B-1 and C preferred stock to common stock	—	—	(39,186,807)	(56,282)	—	—	31,374	56,282	—	—	—	56,282
Issuance of preferred stock and common stock in connection with conversion of debt and accrued interest	15,160,301	6,807	12,138,080	5,450	—	—	849,952	2,188	—	—	—	2,188
Delaware reincorporation	—	—	—	—	—	—	—	(64,168)	64,168	—	—	—
Issuance of special voting stock	—	—	—	—	1	1	—	—	—	—	—	—
Issuance of Series A-1 preferred stock, net of $343 of offering costs	101,263,824	45,124	—	—	—	—	—	—	—	—	—	—
Accretion to redemption value of redeemable convertible preferred stock	—	343	—	—	—	—	—	(252)	(91)	—	—	(343)
Stock-based compensation	—	—	—	—	—	—	—	55	243	—	—	298
Change in fair value of redeemable non-controlling interest	—	—	—	—	—	—	—	—	(154)	—	—	(154)
Consolidated net loss	—	—	—	—	—	—	—	—	—	—	(15,871)	(15,871)
Net loss attributable to redeemable non-controlling interest	—	—	—	—	—	—	—	—	—	—	154	154
Unrealized gain on investment securities	—	—	—	—	—	—	—	—	—	9	—	9
Balance at December 31, 2012	116,424,125	52,274	12,138,080	5,450	1	1	884,179	—	64,166	9	(92,591)	(28,416)
Stock-based compensation	—	—	—	—	—	—	—	—	1,388	—	—	1,388
Change in fair value of redeemable non-controlling interest	—	—	—	—	—	—	—	—	(3,105)	—	—	(3,105)
Share exchange related to non-controlling interest and special voting stock	10,716,405	7,824	—	—	(1)	(1)	—	—	—	—	—	—
Deemed dividend	—	—	—	—	—	—	—	—	(856)	—	—	(856)
Consolidated net loss	—	—	—	—	—	—	—	—	—	—	(20,091)	(20,091)
Net loss attributable to redeemable non-controlling interest	—	—	—	—	—	—	—	—	—	—	96	96
Unrealized loss on investment securities	—	—	—	—	—	—	—	—	—	(7)	—	(7)
Balance at December 31, 2013	127,140,530	$ 60,098	12,138,080	$ 5,450	—	$ —	884,179	$ —	$ 61,593	$ 2	$ (112,586)	$ (50,991)

Celladon Corporation

Consolidated Statements of Preferred Stock and Stockholders' Deficit
(in thousands, except share data)

	Series A-1 Redeemable Convertible Preferred Stock		Convertible Preferred Stock		Special Voting Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2013	127,140,530	60,098	12,138,080	5,450	—	—	884,179	—	61,593	2	(112,586)	(50,991)
Impact of initial public offering												
Initial public offering of common stock, net of $6,342 in offering costs	—	—	—	—	—	—	6,325,000	6	44,252	—	—	44,258
Conversion of convertible notes into common stock	—	—	—	—	—	—	139,644	—	1,117	—	—	1,117
Conversion of convertible preferred stock into common stock	(127,140,530)	(60,098)	(12,138,080)	(5,450)	—	—	11,151,192	11	65,537	—	—	65,548
Warrant liability reclassification	—	—	—	—	—	—	—	—	1,299	—	—	1,299
Common stock issuance upon exercise of warrants	—	—	—	—	—	—	25,481	—	143	—	—	143
Public offering of common stock, net of $3,011 of offering costs	—	—	—	—	—	—	4,600,000	5	40,684	—	—	40,689
Stock-based compensation	—	—	—	—	—	—	—	—	3,319	—	—	3,319
Exercise of stock options	—	—	—	—	—	—	340,220	1	406	—	—	407
Issuance of common stock under employee stock purchase plan	—	—	—	—	—	—	25,021	—	178	—	—	178
Consolidated net loss	—	—	—	—	—	—	—	—	—	—	(33,853)	(33,853)
Unrealized loss on investment securities	—	—	—	—	—	—	—	—	—	(10)	—	(10)
Balance at December 31, 2014	—	$ —	—	$ —	—	$ —	23,490,737	$ 23	$ 218,528	$ (8)	$ (146,439)	$ 72,104

See accompanying notes.

Celladon Corporation

Consolidated Statements of Cash Flows
(in thousands)

	Years Ended December 31,		
	2014	2013	2012
Cash flows from operating activities			
Consolidated net loss	$(33,853)	$(20,091)	$(15,871)
Adjustments to reconcile net loss to net cash used in operating activities			
Depreciation	153	67	64
Stock-based compensation	3,319	1,388	298
Noncash interest expense	388	59	108
Amortization of investment premium	393	255	124
Change in fair value of warrant liability	183	162	—
Loss on disposal of property and equipment	1	—	—
Deferred rent	74	17	28
Changes in operating assets and liabilities:			
Prepaid expenses and other assets	(2,860)	104	(266)
Accounts payable and accrued expenses	2,935	1,843	878
Other liabilities	8	—	—
Net cash used in operating activities	(29,259)	(16,196)	(14,637)
Cash flows from investing activities			
Purchases of investment securities	(90,659)	(17,860)	(26,751)
Proceeds from maturities of investment securities	30,210	28,801	4,966
Purchases of property and equipment	(739)	(87)	(48)
Net cash provided by (used in) investing activities	(61,188)	10,854	(21,833)
Cash flows from financing activities			
Proceeds from issuance of common stock	95,028	—	—
Proceeds from issuance of preferred stock, net	—	—	45,140
Proceeds from issuance of exchangeable shares	—	—	4,814
Proceeds from issuance of convertible debt	—	1,097	—
Repayment of convertible debt	—	—	(111)
Costs paid in connection with common stock offerings	(7,661)	(1,693)	—
Proceeds from borrowing under term loan	10,000	—	—
Costs paid in connection with term loan	(387)	—	—
Other	(1)	—	—
Net cash provided by (used in) financing activities	96,979	(596)	49,843
Net increase (decrease) in cash and cash equivalents	6,532	(5,938)	13,373
Cash and cash equivalents, beginning of period	7,903	13,841	468
Cash and cash equivalents, end of period	$ 14,435	$ 7,903	$ 13,841
Supplemental disclosure of cash flow information			
Interest paid	$ 282	$ —	$ —
Supplemental schedule of noncash investing and financing activities			
Conversion of convertible debt and accrued interest for Series A-1 and Junior preferred and common stock	$ —	$ —	$ 14,430
Share exchange related to non-controlling interest and special voting stock	$ —	$ 7,824	$ —
Deemed dividend	$ —	$ 856	$ —
Accrued purchases of property and equipment	$ 23	$ 166	$ —
Conversion of convertible preferred stock into common stock	$ 65,548	$ —	$ —
Conversion of convertible notes into common stock	$ 1,117	$ —	$ —
Warrant liability reclassification to equity	$ 1,299	$ —	$ —
Capital expenditures funded by capital lease borrowings	$ 12	$ —	$ —

See accompanying notes.

Celladon Corporation

Notes to Consolidated Financial Statements

1. Organization and Summary of Significant Accounting Policies

Organization

Celladon Corporation (Celladon or the Company) was incorporated in California on December 21, 2000 (inception) and reincorporated in Delaware in April 2012. The Company is a clinical-stage biotechnology with industry-leading expertise in the development of cardiovascular gene therapy. The Company applies its leadership position in the field of gene therapy and calcium dysregulation to develop novel therapies for diseases with tremendous unmet medical needs and characterized by an underlying SERCA enzyme deficiency.

As of December 31, 2014, the Company has devoted substantially all of its efforts to product development, raising capital and building infrastructure and has not generated revenues from its planned principal operations.

Use of Estimates

The Company's consolidated financial statements are prepared in accordance with U.S. generally accepted accounting principles (GAAP). The preparation of the Company's consolidated financial statements requires it to make estimates and assumptions that impact the reported amounts of assets, liabilities and expenses and the disclosure of contingent assets and liabilities in the Company's consolidated financial statements and accompanying notes. The most significant estimates in the Company's consolidated financial statements relate to the fair value of equity awards and clinical trial expense accruals. Although these estimates are based on the Company's knowledge of current events and actions it may undertake in the future, actual results may ultimately materially differ from these estimates.

Public Offerings

In February 2014, the Company completed its initial public offering of 6,325,000 shares of common stock at an offering price of $8.00 per share, which included the exercise by the underwriters of their option to purchase 825,000 additional shares of common stock. The Company received net proceeds of $44.3 million after deducting underwriting discounts and commission and offering expenses payable by the Company, including $1.7 million in offering costs paid by the Company prior to December 31, 2013. In connection with the initial public offering, all outstanding shares of convertible preferred stock were converted into shares of common stock, the outstanding principal and accrued interest on the Company's outstanding convertible notes were converted into shares of common stock and the unamortized debt discount related to the convertible notes was charged to expense, warrants to purchase shares of Series A-1 preferred stock were converted into warrants to purchase common stock, the warrant liability was reclassified to additional paid-in capital, and the Company's certificate of incorporation was amended and restated to authorize 200,000,000 shares of common stock and 10,000,000 shares of undesignated preferred stock. See Note 7 for additional information.

In August 2014, the Company completed an underwritten public offering of 4,600,000 shares of common stock at an offering price of $9.50 per share, which included the exercise by the underwriters of their option to purchase 600,000 additional shares of common stock. The Company received net proceeds of $40.7 million after deducting underwriting discounts and commission and offering expenses payable by the Company. See Note 7 for additional information.

Principles of Consolidation

On April 27, 2012, Celladon formed a subsidiary, Celladon Europe B.V. (Celladon Europe), a Dutch limited liability company, for the purpose of managing the new capital investment made by Cooperatief LSP IV U.A. (LSP) related to Celladon's Series A-1 preferred stock (see Note 2). From its inception to June 6, 2013 the subsidiary was 90% owned by Celladon and from June 6, 2013 to December 29, 2014 the subsidiary was wholly

owned by Celladon. Celladon Europe was dissolved on December 30, 2014. The financial statements of Celladon Europe are consolidated with those of the Company. All intercompany transactions and balances were eliminated in consolidation. The U.S. dollar was the functional currency of Celladon Europe. The Company remeasured Celladon Europe's assets and liabilities related to monetary assets and liabilities to the U.S. dollar and recorded the net gains or losses resulting from remeasurement in other income (expense) in the consolidated statements of operations and comprehensive loss. During all periods presented, the Company did not record any material gains or losses from remeasurement.

Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker in making decisions regarding resource allocation and assessing performance. The Company views its operations and manages its business in one operating segment.

Cash and Cash Equivalents

Cash and cash equivalents consists primarily of readily available checking, money market accounts and money market funds. The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents.

Investment Securities

Investment securities primarily consist of investment grade corporate debt securities. The Company classifies all investment securities as available-for-sale. Investments with maturity dates greater than 12 months from the end of each reporting period are classified as long-term. Investment securities are carried at fair value, with the unrealized gains and losses reported as a component of other comprehensive income (loss) in stockholders' equity (deficit) until realized. Realized gains and losses from the sale of investment securities, if any, are determined on a specific identification basis. A decline in the market value of any investment security below cost that is determined to be other than temporary will result in an impairment charge to earnings and a new cost basis for the security is established. No such impairment charges were recorded for any period presented. As of December 31, 2014 and December 31, 2013, none of the investment securities have been in an unrealized loss position for more than 12 months. Premiums and discounts are amortized or accreted over the life of the related security as an adjustment to yield using the straight-line method and are included in interest income. Interest income is recognized when earned.

The following table sets forth the composition of the Company's investment securities (in thousands):

As of December 31, 2014	Maturity in Years	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Corporate debt securities	Less than 1 year	$70,521	$—	$ (8)	$ 70,513

As of December 31, 2013	Maturity in Years	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Corporate debt securities	Less than 1 year	$10,465	$ 2	$ —	$ 10,467

Concentration of Credit Risk

Financial instruments, which potentially subject the Company to significant concentration of credit risk, consist primarily of cash, cash equivalents and investment securities. The Company has established guidelines regarding diversification of investments and their maturities, which are designed to maintain principal and

maximize liquidity. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts and management believes that the Company is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held.

Property and Equipment

Property and equipment are recorded at cost and depreciated using the straight-line method over the estimated useful lives of the assets (generally three to five years) and generally consist of furniture and fixtures, computers, and office equipment. Repairs and maintenance costs are charged to expense as incurred.

Impairment of Long-Lived Assets

Long-lived assets consist primarily of property and equipment. An impairment loss is recorded if and when events and circumstances indicate that assets might be impaired and the undiscounted cash flows estimated to be generated by those assets are less than the carrying amount of those assets. While the Company's current and historical operating losses and negative cash flows are indicators of impairment, management believes that future cash flows to be received support the carrying value of its long-lived assets and, accordingly, has not recognized any impairment losses since inception.

Clinical Trial Accruals

As part of the process of preparing its financial statements, the Company is required to estimate its expenses resulting from its obligations under contracts with vendors and consultants and clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations which vary from contract to contract and may result in payment flows that do not match the periods over which materials or services are provided to us under such contracts. The Company's objective is to reflect the appropriate trial expenses in its financial statements by matching those expenses with the period in which services and efforts are expended. The Company accounts for these expenses according to the progress of the trial as measured by patient progression and the timing of various aspects of the trial. The Company determines accrual estimates through financial models taking into account discussion with applicable personnel and outside service providers as to the progress or state of consummation of trials, or the services completed. During the course of a clinical trial, the Company adjusts its rate of clinical expense recognition if actual results differ from its estimates. The Company makes estimates of its accrued expenses as of each balance sheet date in its financial statements based on the facts and circumstances known at that time. Although the Company does not expect its estimates to be materially different from amounts actually incurred, its understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low for any particular period. Through December 31, 2014, there have been no material adjustments to the Company's prior period estimates of accrued expenses for clinical trials. The Company's clinical trial accrual is dependent upon the timely and accurate reporting of contract research organizations and other third-party vendors.

Preferred Stock Warrant Liability

The Company had issued freestanding warrants to purchase shares of its convertible preferred stock. The fair value of these warrants is classified as a current liability at December 31, 2013 in the accompanying consolidated balance sheets since the underlying redeemable convertible preferred stock is classified as temporary equity at December 31, 2013 in the accompanying consolidated balance sheets instead of in stockholders' equity (deficit) in accordance with authoritative guidance for the classification and measurement of redeemable securities. The warrants were recorded at fair value using the Black-Scholes option pricing model with any changes in fair value being recognized as a component of other income (expense) in the accompanying consolidated statements of operations and comprehensive loss. Upon completion of the Company's initial public offering in February 2014, the warrants no longer required liability accounting and the then fair value of the warrant liability was reclassified into equity.

Deferred Rent

Deferred rent consists of the difference between cash payments and the recognition of rent expense on a straight-line basis for the facility the Company occupies. The Company's lease for its facility provides for fixed increases in minimum annual rental payments. The total amount of rental payments due over the lease term is being charged to rent expense ratably over the life of the lease.

Preferred Stock

The Company classifies preferred stock that is redeemable or subject to liquidation outside of the Company's control outside of permanent equity. For preferred stock that is contractually redeemable outside of the Company's control, the carrying value is increased to its redemption value by accretion in the period of issuance. In the absence of retained earnings, these accretion charges are recorded against additional paid-in capital.

Research and Development Costs

Research and development expenses consist primarily of salaries and related overhead expenses; fees paid to consultants and contract research organizations; costs related to acquiring and manufacturing clinical trial materials; costs related to compliance with regulatory requirements; and maintenance and license payments related to licensed product candidates and technologies. All research and development costs are expensed as incurred.

Patent Costs

Costs related to filing and pursuing patent applications are recorded as general and administrative expense and expensed as incurred since recoverability of such expenditures is uncertain.

Stock-Based Compensation

Stock-based compensation expense represents the cost of the grant date fair value of employee stock option grants and stock purchases under the Employee Stock Purchase Plan (ESPP) recognized over the requisite service period of the awards (usually the vesting period) on a straight-line basis, net of estimated forfeitures. The Company estimates the fair value of the awards using the Black-Scholes option pricing model.

The Company accounts for stock options granted to non-employees using the fair value approach. These option grants are subject to periodic revaluation over their vesting terms.

Income Taxes

The Company accounts for income taxes under the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements. Under this method, deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. The effect of a change in tax rates on deferred tax assets and liabilities is recognized in income in the period that includes the enactment date.

The Company recognizes net deferred tax assets to the extent that management believes these assets are more likely than not to be realized. In making such a determination, management considers all available positive and negative evidence, including future reversals of existing taxable temporary differences, projected future taxable income, tax-planning strategies, and results of recent operations. If management determines that the Company would be able to realize its deferred tax assets in the future in excess of their net recorded amount, management would make an adjustment to the deferred tax asset valuation allowance, which would reduce the provision for income taxes.

The Company records uncertain tax positions on the basis of a two-step process whereby (1) management determines whether it is more likely than not that the tax positions will be sustained on the basis of the technical merits of the position and (2) for those tax positions that meet the more-likely-than-not recognition threshold, management recognizes the largest amount of tax benefit that is more than 50% likely to be realized upon ultimate settlement with the related tax authority. The Company recognizes interest and penalties related to unrecognized tax benefits within income tax expense. Any accrued interest and penalties are included within the related tax liability.

Comprehensive Loss

Comprehensive loss is defined as a change in equity during a period from transactions and other events and circumstances from non-owner sources. The Company's only component of other comprehensive loss is unrealized gains (losses) on investment securities. Comprehensive loss has been reflected in the consolidated statements of operations and comprehensive loss and as a separate component of the statements of stockholders' deficit for all periods presented.

Net Loss Per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method. Dilutive common stock equivalents are comprised of convertible preferred stock and rights to acquire convertible preferred stock (non-controlling interest), warrants for the purchase of common stock and options outstanding under the Company's stock option plans. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding due to the Company's net loss position.

Potentially dilutive securities not included in the calculation of diluted net loss per share attributable to common stockholders because to do so would be anti-dilutive are as follows (in common stock equivalent shares):

	Years Ended December 31,	
	2014	2013
Redeemable convertible preferred stock	—	10,179,372
Convertible preferred stock	—	971,820
Warrants for convertible preferred stock	—	231,821
Warrants for common stock	206,340	702
Common stock options	2,408,634	1,543,667
	2,614,974	12,927,382

Recent Accounting Pronouncements

In July 2013, the FASB issued Accounting Standards Update (ASU) No. 2013-11, *Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists*. ASU 2013-11 provides explicit guidance on the financial statement presentation of an unrecognized tax benefit when a net operating loss carryforward, a similar tax loss, or a tax credit carryforward exists. The guidance is effective prospectively for fiscal years, and interim periods within those years, beginning after December 15, 2013, with an option for early adoption. On January 1, 2014, the Company adopted this standard, which had no impact on its financial position or results of operations.

In June 2014, the FASB issued Accounting Standards Update (ASU) No. 2014-10, *Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*. ASU 2014-10 a) eliminates the requirement for development stage entities to present inception-to-date information in the statements of income, cash flows and shareholder equity, b) amends Topic 275 to clarify that the risk and uncertainty disclosure requirements apply to entities that have not commenced principal operations, c) eliminates the exception related to the sufficiency of equity at risk for development stage entities from the guidance on variable interest entities in paragraph 810-10-15-16 to increase consistency in application of consolidated guidance across all entities and d) removes the definition of *development stage entities* from the Master Glossary of the Accounting Standards Codification. The amendments in this Update are to be applied retrospectively except for the clarification to Topic 275, which shall be applied prospectively. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2014, with an option for early adoption. The Company adopted this guidance prior to filing this Annual Report on Form 10-K for the year ended December 31, 2014. The adoption of ASU 2014-10 impacted disclosure only and did not have any impact on the Company's financial position or results of operations.

In June 2014, the FASB issued ASU No. 2014-12, *Accounting for Share-Based Payments When the Terms of an Award Provide That a Performance Target Could Be Achieved after the Requisite Service Period*, which clarifies that entities should treat performance targets that can be met after the requisite service period of a share-based payment award as performance conditions that affect vesting. This standard is effective for annual reporting periods ending after December 15, 2015 and interim periods within those annual periods. Early adoption is permitted. The adoption of this guidance is expected to have no impact on the Company's financial position or results of operations.

In August 2014, the FASB issued ASU No. 2014-15, *Presentation of Financial Statements Going Concern*, which requires management to assess an entity's ability to continue as a going concern, and to provide related footnote disclosure in certain circumstances. This standard is effective for annual reporting periods ending after December 15, 2016 and interim periods thereafter. Early adoption is permitted. The adoption of this guidance is expected to have no impact on the Company's financial position or results of operations.

In November 2014, the FASB issued ASU No. 2014-16, *Determining Whether the Host Contract in a Hybrid Financial Instrument Issued in the Form of a Share is More Akin to Debt or Equity*, which requires the use of the whole instrument approach in determining the nature of a host contract in a hybrid instrument. This standard is effective for annual reporting periods ending after December 15, 2015 and interim periods within those annual periods. Early adoption is permitted. The adoption of this guidance is expected to have no impact on the Company's financial position or results of operations.

2. Celladon Europe B.V.

In April 2012 and June 2012, LSP invested an aggregate of $4.8 million in Celladon Europe. In exchange for the investment, the Company issued LSP one share of Special Preferred Voting stock and Celladon Europe issued LSP 1,999 non-voting B shares. The 1,999 B shares were exchangeable into 10,716,405 shares of the Company's Series A-1 preferred stock at the option of LSP. The Company determined that the investment held by LSP in Celladon Europe should be classified as a redeemable non-controlling interest, as the shares of Celladon Europe were not in-substance common stock. In-substance common stock is an investment in an entity that has risk and reward characteristics that are substantially similar to that entity's common stock. Due to the liability characteristics associated with the shares of Celladon Europe held by LSP, the Company concluded that the investor's shares were not substantially similar to common stock. The liability characteristics included the investor's put rights, which provided the investor with the ability to exchange its shares in Celladon Europe for Series A-1 preferred stock of the Company.

The redeemable non-controlling interest was initially valued using the fair value of the Series A-1 preferred stock. At each reporting period, the Company adjusted the carrying value of the redeemable non-controlling interest by the net loss attributable to the redeemable non-controlling interest. Any difference between the fair

value and the adjusted carrying value of the redeemable non-controlling interest was recorded as an adjustment to additional paid-in capital and presented as a component of net loss attributable to common stockholders in the accompanying consolidated statements of operations and comprehensive loss.

From April 2012 through June 6, 2013, LSP owned approximately 10% of Celladon Europe.

On June 6, 2013, LSP delivered a notice to exchange its 1,999 B shares of Celladon Europe for 10,716,405 shares of the Company's Series A-1 preferred stock. Concurrently, the one share of outstanding Special Preferred Voting stock was cancelled. As of June 6, 2013, the redeemable non-controlling interest was adjusted to fair value and reclassified to Series A-1 preferred stock on the accompanying consolidated balance sheet.

During the years ended December 31, 2013 and 2012, the Company adjusted the loss attributable to common stockholders as a result of increases in the fair value of the redeemable non-controlling interest of approximately $3.1 million and $0.2 million, respectively. The increases in fair value increased the loss attributable to common stockholders.

In May 2014, the Company completed the transfer of the open clinical site contracts from Celladon Europe to Celladon and on December 30, 2014, Celladon Europe was dissolved. Upon dissolution, final administrative fees were settled and cash of approximately $0.1 million was transferred from Celladon Europe to Celladon.

3. Balance Sheet Details

Property and equipment consist of the following (in thousands):

	As of December 31,	
	2014	2013
Office furniture and other equipment	$ 881	$ 555
Leasehold improvements	246	—
Accumulated depreciation	(364)	(247)
	$ 763	$ 308

Accounts payable and accrued expenses consist of the following (in thousands):

	As of December 31,	
	2014	2013
Accounts payable	$3,293	$1,397
Accrued compensation	1,909	664
Accrued other	596	839
Current portion of deferred rent	5	8
	$5,803	$2,908

4. Fair Value Measurements

The Company's financial instruments primarily consist of cash and cash equivalents, investment securities, accounts payable and accrued liabilities. The carrying value of these financial instruments generally approximates fair value due to their short-term nature. Investment securities, warrant liabilities and redeemable non-controlling interest are recorded at fair value. Based on the borrowing rates currently available to the Company for loans with similar terms, which is considered a Level 2 input, the Company believes that the fair value of its convertible debt approximates its carrying value.

The accounting guidance defines fair value, establishes a consistent framework for measuring fair value and expands disclosure for each major asset and liability category measured at fair value on either a recurring or nonrecurring basis. Fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a basis for considering such assumptions, the accounting guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value as follows:

Level 1: Observable inputs such as quoted prices in active markets;

Level 2: Inputs, other than the quoted prices in active markets, that are observable either directly or indirectly; and

Level 3: Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions

As of December 31, 2014 and 2013, cash and cash equivalents consist primarily of bank deposits with third-party financial institutions and highly liquid money market securities with original maturities at date of purchase of 90 days or less and are stated at cost which approximate fair value and are classified within the Level 1 designation discussed above. Marketable securities are recorded at fair value, defined as the exit price in the principal market in which the Company would transact, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. Level 2 securities are valued using quoted market prices for similar instruments, non-binding market prices that are corroborated by observable market data, or discounted cash flow techniques and include the Company's investments in corporate debt securities and commercial paper. Financial assets and liabilities that are measured or disclosed at fair value on a recurring basis, and are classified within the Level 3 designation, include the warrant liability and redeemable non-controlling interest. None of the Company's non-financial assets and liabilities are recorded at fair value on a non-recurring basis. No transfers between levels have occurred during the periods presented.

Cash equivalents measured at fair value as of December 31, 2014 and 2013, are all classified within Level 1. Below is a summary of assets and liabilities measured at fair value (in thousands):

		Fair Value Measurements at Reporting Date Using		
	As of December 31, 2014	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Assets:				
Corporate debt securities	$ 70,513	$ —	$ 70,513	$ —

		Fair Value Measurements at Reporting Date Using		
	As of December 31, 2013	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Assets:				
Corporate debt securities	$ 10,467	$ —	$ 10,467	$ —
Liabilities:				
Convertible notes	$ 1,044	—	—	$ 1,044
Warrant liability	1,116	—	—	1,116
	$ 2,160	$ —	$ —	$ 2,160

The Company determined the fair value of the convertible notes utilizing an estimated cost of debt for comparable venture backed and mezzanine financings.

The fair value per share of the Company's underlying Series A-1 preferred stock was used to determine the fair value of the redeemable non-controlling interest and the warrant liability. As of February 4, 2014, December 31, 2013, October 15, 2013 (issuance date of Series A-1 warrants), June 6, 2013 (exchange date of exchangeable shares) and December 31, 2012, the fair value of the Series A-1 preferred stock was $0.64, $0.64, $0.91, $0.73 and $0.449, respectively. The fair value of the Series A-1 preferred stock was determined using either an option pricing model, a hybrid option pricing and probability weighted expected return model or, in the case of the February 4, 2014 and December 31, 2013 values, derived from the Company's IPO price. The key inputs into the models included the probability and timing of expected liquidity event dates, discount rates and the selection of appropriate market comparable transactions and multiples to apply to the Company's various historical and forecasted operational metrics.

In addition to the fair value of the underlying Series A-1 preferred stock, the following assumptions were used in the Black-Scholes option pricing model to determine the fair value of the preferred stock warrant liability:

	October 15, 2013	December 31, 2013	February 4, 2014
Risk-free interest rate	1.37%	1.58%	1.58%
Expected volatility	79%	82%	82%
Expected term (in years)	5.0	4.8	4.7
Expected dividend yield	0.0%	0.0%	0.0%

The following table provides a reconciliation of all liabilities measured at fair value using Level 3 significant unobservable inputs (in thousands):

	Redeemable Non-Controlling Interest	Convertible Notes	Warrant Liability
Balance at December 31, 2011	$ —	$ —	$ —
Issuance of shares of redeemable non-controlling interest	4,814	—	—
Net loss attributable to redeemable non-controlling interest	(154)	—	—
Change in fair value	154	—	—
Balance at December 31, 2012	4,814	—	—
Issuance of warrants in connection with note and warrant purchase agreement	—	—	954
Issuance of debt	—	999	—
Net loss attributable to redeemable non-controlling interest	(96)	—	—
Changes in fair value	3,105	45	162
Exchange of redeemable non-controlling interest for Series A-1 preferred stock	(7,823)	—	—
Balance at December 31, 2013	—	1,044	1,116
Changes in fair value	—	53	183
Reclassification to equity upon initial public offering	—	—	(1,299)
Conversion to common stock upon initial public offering	—	(1,097)	—
Balance at December 31, 2014	$ —	$ —	$ —

5. Commitments and Contingencies

Note and Warrant Purchase Agreement

In October 2013, the Company entered into a note and warrant purchase agreement with certain existing investors for the sale of up to an aggregate of $1,097,017 of convertible promissory notes (the 2013 Notes) and warrants exercisable to purchase shares of Series A-1 Preferred Stock (the 2013 Warrants).

The terms of the 2013 Notes provided for their automatic conversion (including accrued interest) upon a qualified initial public offering or private placement of equity securities into common stock or other equity securities issued in such private placement at a conversion price per share equal to the initial public offering price or per share purchase price to investors in the private placement. The conversion of the 2013 Notes in the event of a qualified initial public offering or private placement of equity was deemed to be the predominant settlement mechanism. As this predominant settlement mechanism provided for the settlement of a fixed monetary amount in a variable number of equity instruments, the Company concluded that it was appropriate to recognize the 2013 Notes at fair value. The Company valued the 2013 Notes utilizing an estimated cost of debt for comparable venture backed and mezzanine financings. The initial fair value of the 2013 Notes was approximately $1.0 million. Upon completion of the Company's initial public offering in February 2014, the 2013 Notes plus approximately $20,000 of accrued interest automatically converted into 139,644 shares of common stock.

The 2013 Warrants were initially accounted for as liabilities with subsequent changes in fair value recognized within the consolidated statement of operations. The Company determined that the initial fair value of the 2013 Warrants was $1.0 million. The fair value of the 2013 Warrants was derived from the probability weighted expected return model the Company used to value its common stock. Upon completion of the Company's initial public offering in February 2014, the warrants no longer require liability accounting and the then fair value of the warrant liability was reclassified into equity. The warrants also became exercisable for an aggregate of 231,821 shares of common stock at an exercise price of $5.61 per share.

The initial recognition of the 2013 Notes and 2013 Warrants at fair value resulted in a deemed dividend in the amount of $0.9 million that was accounted for as additional net loss attributable to common stockholders.

Sublicense Agreement and Amended and Restated License Agreement with AmpliPhi

Sublicense Agreement

In June 2012, the Company entered into a sublicense agreement (the AmpliPhi Sublicense) with AmpliPhi Biosciences Corporation (AmpliPhi), pursuant to which AmpliPhi sublicensed to the Company certain rights under a separate agreement which AmpliPhi entered into in 2009 with the Trustees of University of Pennsylvania (UPenn). Under the terms of the AmpliPhi Sublicense, the Company obtained an exclusive, worldwide sublicense from AmpliPhi under certain UPenn patents related to AAV1 vectors for the development, manufacture, use and sale of companion diagnostics to MYDICAR. In addition, the Company is required to use commercially reasonable efforts to meet certain developmental, regulatory and commercial milestones with respect to companion diagnostics under the agreement. The Company is currently in compliance with these milestone requirements. In consideration for the sublicense granted to the Company under the agreement, the Company paid to AmpliPhi a sublicense initiation fee of $310,000, and the Company is obligated to pay to AmpliPhi an annual sublicense maintenance fee of $310,000. The Company is also required to pay to AmpliPhi a low single-digit percentage royalty based on net sales of any companion diagnostic covered by a licensed patent sold by the Company, its affiliates or its sublicensees. The Company's royalty obligations continue on a companion diagnostic-by-companion diagnostic and country-by-country basis until the expiration of the last-to-expire valid claim in a licensed patent covering the applicable companion diagnostic in such country. Finally, the Company is obligated to pay to AmpliPhi all royalty and milestone payments that become due and payable by AmpliPhi to UPenn under AmpliPhi's agreement with UPenn as a result of the Company's exercise of the sublicense granted under the Company's agreement with AmpliPhi, including a low single-digit tiered percentage royalty on net sales of any companion diagnostic sold by the Company, its affiliates or its sublicensees, which royalty is separate from and in addition to the royalty payable to AmpliPhi described above, and up to an aggregate of $850,000 in potential milestone payments per product covered by the licensed patents.

The Company may unilaterally terminate the agreement upon 30 days' written notice to AmpliPhi. Absent early termination, the agreement will automatically terminate upon the expiration of the last-to-expire licensed patent, which is expected to be in 2019.

The Company has recorded research and development expense related to sublicense fees under the agreement of $0.3 million, $0.3 million and $0.3 million, respectively, for the years ended December 31, 2014, 2013 and 2012. Through December 31, 2014, no milestone obligations were incurred under the agreement.

Amended and Restated License Agreement

The Company entered into an amended and restated license agreement with AmpliPhi concurrently with the AmpliPhi Sublicense that both amended the terms of the license agreement which the Company entered into with AmpliPhi in 2009 and terminated its manufacturing agreement with AmpliPhi which the Company entered into in 2009. Under the agreement, the Company obtained an exclusive, worldwide license under certain patents and know-how related to AmpliPhi's AAV vector and manufacturing technology for the development, manufacture, use and sale of MYDICAR. In addition, the Company has agreed to use commercially reasonable efforts to meet certain diligence milestones with respect to the development and commercialization of at least one product covered by the UPenn patent rights licensed to AmpliPhi by UPenn under the Company's agreement with UPenn.

The Company is currently in compliance with these milestone requirements. During the term of the agreement, the Company is not obligated to make annual license or maintenance payments, but is obligated to pay to AmpliPhi all royalty and milestone payments that become due and payable by AmpliPhi to UPenn under AmpliPhi's agreement with UPenn as a result of the Company's exercise of the sublicense granted under the Company's agreement with AmpliPhi. This includes a low single-digit tiered percentage royalty on net sales of MYDICAR and any other product covered by the licensed patents sold by the Company, its affiliates or its sublicensees, and up to $850,000 in milestone payments upon the achievement of certain developmental and regulatory milestones related to MYDICAR and any other product covered by the licensed patents. Through December 31, 2014, no milestone obligations were incurred under the agreement. The agreement does not provide either party with termination rights and does not have a provision for expiration or automatic termination. In addition, the Company paid $3.2 million in exchange for certain intangible assets associated with the license agreement that the Company acquired from AmpliPhi in June 2012, which were expensed as in-process research and development during the year ended December 31, 2012.

Exclusive Patent License with the Regents of the University of Minnesota

In May 2009, the Company entered into an exclusive patent license agreement with the Regents of the University of Minnesota (UMinn) under which it obtained an exclusive license to UMinn's joint ownership interest in a patent application related to screening technology for isolation of small molecule modulators of SERCA enzymes. The agreement does not encompass a manufacturing agreement.

The Company has agreed to meet certain performance milestones under the agreement, the deadline for which may be extended at the Company's request provided that the Company has used commercially reasonable efforts to achieve such milestones by the applicable deadlines. The Company is currently in compliance with these milestone requirements. The Company has the first right to prosecute and maintain the applicable patent family.

The Company made an upfront payment to UMinn of $120,000. In addition, the Company is obligated to pay to UMinn an annual license fee of $120,000. The annual license fee will increase to $325,000 if the Company (1) undergoes a change of control, (2) assigns the agreement, any of its rights or obligations under the agreement or as joint ownership interest in the licensed technology, (3) receives a certain amount in license and sublicense revenues under the agreement, (4) files an investigational new drug application, or IND, new drug application, biologic license application or orphan drug application (or a foreign equivalent of any such application) for a product covered by the licensed technology, or (5) enters into any agreement with a third party to market or use the licensed technology, subject to certain exceptions.

The Company may unilaterally terminate the agreement upon 90 days' written notice to UMinn. UMinn may terminate the agreement upon 10 days' written notice to the Company upon the Company's insolvency or for its breach of the agreement if such breach remains uncured for 90 days after the Company receives notice of such breach, or 30 days in the case of a non-payment breach. Absent early termination, the agreement will automatically terminate upon the expiration of all active claims in any licensed patent or patent application, which is expected to occur no earlier than January 2030.

The Company has recorded research and development expense related to license and annual maintenance fees under the agreement of $0.1 million, $0.1 million, and $0.1 million, respectively, for the years ended December 31, 2014, 2013 and 2012. Through December 31, 2014, no milestone obligations were incurred under the agreement.

Material Transfer and Exclusivity Agreement

In February 2014, the Company and Les Laboratoires Servier (Servier) entered into a material transfer and exclusivity agreement, pursuant to which the Company agreed to transfer to Servier samples of certain proprietary compounds from the Company's small molecule SERCA2b modulator program and granted to Servier a non-exclusive, non-sublicensable, royalty-free license to conduct certain studies of the samples for the purpose of evaluating Servier's interest in negotiating a potential license and research collaboration agreement with the Company relating to small molecule SERCA2b modulators (Compounds), for the treatment of type 2 diabetes and other metabolic diseases.

Although the evaluation period under this Agreement has expired, the Company is in the process of completing certain pre-clinical studies of these compounds in coordination with Servier and Servier is continuing to evaluate its potential interest in this program.

Under the terms of the agreement, the Company also granted to Servier the exclusive right to negotiate for an exclusive, royalty-bearing license to develop and commercialize Compounds, and products containing Compounds, in the field of type 2 diabetes and other metabolic diseases, or the field, solely outside of the United States and its territories and possessions on the terms and conditions set forth in the agreement and other commercially reasonable terms to be negotiated in good faith by the parties and set forth in a definitive license and research collaboration agreement.

License Agreement with Enterprise

On July 18, 2014, the Company and Enterprise Partners Management, LLC (Enterprise), an affiliate of Enterprise Partners Venture Capital, entered into an Assignment and License Agreement (the Enterprise License Agreement), pursuant to which Enterprise granted to the Company an exclusive, worldwide license and the assignment of patents held by Enterprise relating to certain gene therapy applications of the membrane-bound form of the Stem Cell Factor gene (mSCF) for treatment of cardiac ischemia. The Company has the right to grant sublicenses to third parties under the Enterprise License Agreement. Entities affiliated with Enterprise beneficially owned more than 10% of Celladon's stock as of the date the Enterprise License Agreement was executed.

In consideration for the rights granted to the Company under the Enterprise License Agreement, the Company paid an upfront fee to Enterprise of $160,000. The Company is also obligated to pay to Enterprise a milestone payment in the amount of $1,000,000 upon the grant to the Company, a Company affiliate or a Company sublicensee of the first regulatory approval in the United States of a product that is covered by the licensed patents. In addition, the Company is required to pay to Enterprise a 2% royalty on net sales of products sold by the Company, Company affiliates and Company sublicensees that are covered by the licensed patents. The Company's royalty obligations continue on a product-by-product and country-by-country basis until the expiration of the last-to-expire valid claim in the licensed patents covering a licensed product in such country.

The Company may unilaterally terminate the Enterprise License Agreement upon written notice to Enterprise. Enterprise may terminate the agreement in the event of the Company's material breach of the Enterprise License Agreement if such breach remains uncured for 90 days following receipt of written notice of such breach. Absent early termination, the Enterprise License Agreement will automatically terminate upon the expiration of the last-to-expire of the licensed patents containing a valid claim.

Other License Agreements

The Company has entered into various license agreements pursuant to which the Company acquired certain intellectual property. Pursuant to each agreement the Company paid a license fee and reimbursed historical patent costs. Additionally, under each agreement, the Company may be required to pay annual maintenance fees, royalties, milestone payments and sublicensing fees. Each of the license agreements is generally cancelable by the Company, given appropriate prior written notice. Minimum annual payments to maintain these cancelable licenses total an aggregate of approximately $0.2 million and potential future milestone payments total an aggregate of approximately $3.3 million. The Company has recorded research and development expense related to license and annual maintenance fees under the agreements of $0.2 million for each of the years ended December 31, 2014, 2013 and 2012.

Through December 31, 2014, the Company has recorded research and development expense of $0.1 million related to milestone obligations incurred under the agreements.

Leases

The Company leases office space in San Diego, California under long-term operating leases that expire in October 2017 and September 2021. On July 1, 2014, the Company relocated its San Diego office to another location in San Diego and is subleasing the prior space. The Company also has short-term leases for satellite office space in Seattle, Washington and housing accommodation in San Diego that expire in 2015. Rent expense was $0.3 million, $0.1 million and $0.1 million for the years ended December 31, 2014, 2013 and 2012, respectively. In March 2015, the Company entered into a short-term lease for approximately 7,000 additional square feet of office space in Seattle, Washington that expires in June 2016.

The future minimum annual rental commitments under the lease obligations and sublease rental receipts at December 31, 2014 are as follows (in thousands):

	Lease Obligations	Sublease Rental Receipts	Total
Year ending December 31:			
2015	$ 466	$ (75)	$ 391
2016	487	(77)	410
2017	485	(67)	418
2018	414	—	414
2019	426	—	426
Thereafter	776	—	776
Total	$ 3,054	$ (219)	$2,835

6. Long-Term Obligations

Hercules Loan Agreement

On July 31, 2014, the Company entered into a Loan and Security Agreement (the Loan Agreement) with Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc. (as agent and as a lender, and together with Hercules Technology III, L.P., the Lenders) under which the Company may borrow up to $25.0 million in two tranches (the Loan).

The Company borrowed the first tranche of $10.0 million on August 1, 2014 and paid a facility charge to the Lenders of $150,000 in addition to $37,500 previously paid to the Lenders as a commitment fee. The Company plans to use the proceeds of the Loan to provide additional funding for the development of MYDICAR, for other development programs in its pipeline and for general corporate purposes. The second tranche of up to $15.0 million can be drawn through June 30, 2015 (amended from May 31, 2015), but only if the Company has provided the Lenders with notice that data from the Company's Phase 2b clinical trial for MYDICAR supports the continued development of MYDICAR for its Breakthrough Therapy designation to either a Phase 3 clinical trial or for registration for approval, as reasonably determined by the Company's senior management and board of directors (the Milestone). Upon funding of the second tranche of the Loan, the Company will be required to pay a facility charge to the Lenders of $100,000.

The interest rate for each tranche will be calculated at a rate equal to the greater of either (i) 8.25% plus the prime rate as reported from time to time in The Wall Street Journal minus 5.25%, and (ii) 8.25%. Payments under the Loan Agreement are interest only until August 1, 2015 (which will be extended until February 1, 2016 if the Company achieves the Milestone on or before June 30, 2015) (the Amortization Date) followed by equal monthly payments of principal and interest through the scheduled maturity date on February 1, 2018 (the Loan Maturity Date). In addition, a final payment equal to $1,750,000 will be due at such time as the Loan is prepaid or becomes due and payable as specified in the Loan Agreement. The Company's obligations under the Loan Agreement are secured by a security interest in substantially all of its assets, excluding its intellectual property but including the proceeds from the sale, licensing or disposition of its intellectual property. The Company's intellectual property is also subject to customary negative covenants.

If the Company prepays the loan prior to maturity, it will pay the Lenders a prepayment charge, based on a percentage of the then outstanding principal balance, equal to 1.5% if the prepayment occurs prior to the Amortization Date.

Subject to certain conditions and limitations set forth in the Loan Agreement, including ownership limitations of the Lenders, the Company has the right to convert up to $3.0 million of scheduled principal installments of the Loan into shares of the Company's common stock, provided such shares must be freely tradable. The number of shares of common stock that would be issued upon conversion would be equal to the number determined by dividing (x) the principal amount to be paid in shares of common stock by (y) $16.33.

The Loan Agreement includes customary representations, warranties and covenants (affirmative and negative) of the Company, including restrictive covenants that limit the Company's ability to: incur additional indebtedness; encumber the collateral securing the Loan; acquire, own or make investments; repurchase or redeem stock or other equity securities; declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest; transfer a material portion of the Company's assets; acquire other businesses; and merge or consolidate with or into any other business organization. The Loan Agreement does not however include any financial maintenance covenants. The Loan Agreement also includes standard events of default, including payment defaults, breaches of covenants following any applicable cure period, a material impairment in the perfection or priority of the Lenders' security interest or in the value of the collateral, and events relating to bankruptcy or insolvency. Upon the occurrence of an event of default, a default interest rate of an additional 5% may be applied to the outstanding Loan, and the Lenders may declare all outstanding obligations immediately due and payable and take such other actions as are set forth in the Loan Agreement.

Capital Lease

In 2014 the Company entered into a capital lease arrangement for office equipment in the Company's San Diego, California office.

Contractual Payments and Carrying-Value Reconciliation

As of December 31, 2014, future contractual principal and final fee payments on the Company's debt and capital lease obligations are as follows (in thousands):

Year ending December 31:	Total	Term Loan	Capital Lease
2015	$ 1	$ —	$ 1
2016	3,432	3,430	2
2017	4,052	4,049	3
2018	4,275	4,271	4
2019	2	—	2
Total	$11,762	$11,750	$ 12

The following table provides a reconciliation of our future contractual principal and final fee payments on our debt and capital lease obligations to the reported carrying value as of December 31, 2014 (in thousands):

Total loan debt and capital lease obligations	$11,762
Less: Debt discount	(1,659)
Total carrying value:	10,103
Less: Carrying value of current portion of long-term obligations	(1)
Carrying value of long-term obligations, less current portion	10,102

Interest expense for the years ended December 31, 2014, 2013 and 2012 was $0.7 million, $0.1 million and $0.1 million, respectively. Interest expense in 2014 related mainly to the Hercules Loan Agreement and interest expense in prior years related mainly to convertible debt outstanding in those periods.

7. Preferred Stock and Stockholders' Equity (Deficit)

Preferred Stock

In addition to its redeemable convertible preferred stock, the Company's convertible preferred stock has been classified as temporary equity at December 31, 2013 on the accompanying consolidated balance sheets instead of in stockholders' deficit in accordance with authoritative guidance for the classification and measurement of potentially redeemable securities whose redemption is based upon certain change in control events that are outside of the control of the Company, including liquidation, sale or transfer of control of the Company. On February 4, 2014, in connection with the Company's initial public offering, all outstanding shares of convertible preferred stock were converted into 11,151,192 shares of the Company's common stock.

The authorized, issued and outstanding shares of preferred stock by series are as follows (in thousands, except share amounts):

	Shares Authorized	Shares Outstanding	Liquidation Preference	Redemption Amount
As of December 31, 2014				
Preferred stock	10,000,000	—	—	—
As of December 31, 2013				
Redeemable convertible preferred stock:				
Series A-1	135,826,497	127,140,530	$ 114,172	$ 57,086
Convertible preferred stock:				
Junior preferred stock	12,138,080	12,138,080	5,450	—
Total	147,964,577	139,278,610	$ 119,622	$ 57,086

Common Stock and Common Stock Warrants

In February 2014, the Company completed its initial public offering in which it sold 6,325,000 shares of common stock at a public offering price of $8.00 per share.

The proceeds received and costs incurred in connection with the Company's initial public offering, shown in the period received or paid, were as follows (in thousands):

		Year ended December 31,	
	Total	2014	2013
Gross proceeds (including over-allotment)	$50,600	$ 50,600	$ —
Underwriting discounts and commissions	(3,542)	(3,542)	—
Offering costs	(2,800)	(1,107)	(1,693)
Net proceeds	$44,258	$ 45,951	$ (1,693)

In addition, each of the following occurred on February 4, 2014 in connection with the Company's initial public offering:

- Series A-1 redeemable convertible preferred stock outstanding (127,140,530 shares) and Junior preferred convertible stock outstanding (12,138,080 shares) were converted into 10,179,372 and 971,820 shares of the Company's common stock, respectively;
- the outstanding principal balance of $1,097,017 and accrued interest of $20,000 on convertible promissory notes converted into 139,644 shares of the Company's common stock;
- warrants to purchase 2,895,570 shares of Series A-1 preferred stock were converted into warrants to purchase 231,821 shares of the Company's common stock and the warrant liability was reclassified to additional paid-in capital.

In August 2014, the Company completed an underwritten public offering in which it sold 4,600,000 shares of common stock at a public offering price of $9.50 per share. The proceeds received and costs incurred in connection with this offering, shown in the period received or paid, were as follows (in thousands):

	2014
Gross proceeds (including option to purchase additional shares)	$43,700
Underwriting discounts and commissions	(2,622)
Offering costs	(389)
Net proceeds	$40,689

The following table summarizes the fully exercisable warrants outstanding for the purchase of common stock as of December 31, 2014 and 2013:

December 31,		Exercise	Expiration
2014	2013	Price	Date
—	80	$ 224.82	January 2015
—	622	$ 12.49	October 2016
206,340	—	$ 5.61	October 2018
206,340	702		

Stock Options

Options granted under the Company's equity incentive plans generally expire no more than 10 years from the date of grant and generally vest and become exercisable over a period not to exceed four years, as determined by the Company's board of directors. Recipients of stock options are eligible to purchase shares of the Company's common stock at an exercise price equal to no less than the estimated fair market value of such stock on the date of grant.

Prior Plans

In December 2001, the Company adopted its 2001 Stock Option Plan (the 2001 Plan) and in January 2012 adopted its 2012 Equity Incentive Plan (the 2012 Plan, and together with the 2001 Plan, the Prior Plans). The Prior Plans have terminated and no further shares may be granted under the Prior Plans.

2013 Equity Incentive Plan

In October 2013, the Company's stockholders approved the 2013 Equity Incentive Plan, as amended (2013 Plan), which became effective in February 2014. Under the 2013 Plan, the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units, performance-based stock awards and other awards to individuals who are then employees, officers, non-employee directors or consultants of the Company and its affiliates. Additionally, the 2013 Plan provides for the grant of performance cash awards. Initially, the aggregate number of shares of common stock that may be issued pursuant to stock awards under the 2013 Plan is the sum of (1) 1,473,738 shares, plus (2) the number of shares (not to exceed 1,569,905 shares) (i) the 26,294 shares reserved for issuance under the 2012 Plan at the time the 2013 Plan became effective, and (ii) any shares subject to outstanding stock options or other stock awards that were granted under the 2012 Plan or 2001 Plan that are forfeited, terminate, expire or are otherwise not issued. Additionally, the number of shares of common stock reserved for issuance under the 2013 Plan will automatically increase on January 1 of each year, beginning on January 1, 2015 and continuing through and including January 1, 2023, by 5% of the total number of shares of the Company's capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by the Company's board of directors.

A summary of the Company's stock option activity under the Prior Plans and 2013 Plan is as follows:

	Options	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term	Aggregate Intrinsic Value (in 000's)
Outstanding at December 31, 2013	1,543,667	$ 3.19	8.66	$ 9,128
Granted	1,240,673	10.61		
Exercised	(340,220)	1.20		
Canceled	(35,486)	10.93		
Outstanding at December 31, 2014	2,408,634	$ 7.18	8.53	$ 31,031
Options exercisable at December 31, 2014	1,263,695	$ 4.02	7.78	$ 20,877
Options exercisable, vested and expected to vest at December 31, 2014	2,408,634	$ 7.18	8.53	$ 31,031

The weighted-average grant date fair value of employee options granted during the years ended December 31, 2014, 2013 and 2012 was $7.36, $7.27 and $0.75 per share, respectively. The aggregate intrinsic value of options exercised during the year ended December 31, 2014 was approximately $3.9 million. There were no options exercised in the years prior to the Company's initial public offering in 2014.

2013 Employee Stock Purchase Plan

In October 2013, the Company's stockholders approved the 2013 Equity Stock Purchase Plan (ESPP) which became effective in February 2014. Initially, the ESPP authorizes the issuance of 165,732 shares of common stock pursuant to purchase rights granted to the Company's employees or to employees of any of the Company's designated affiliates. The number of shares of common stock reserved for issuance will automatically increase on January 1 of each calendar year, from January 1, 2015 through January 1, 2023 by the least of (1) 1% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year, (2) 384,307 shares, or (3) a number determined by the Company's board of directors that is less than (1) and (2). During the year ended December 31, 2014, we recorded stock-based compensation expense of approximately $0.1 million related to the ESPP.

Stock-Based Compensation Expense

The weighted-average assumptions used in the Black-Scholes option pricing model to determine the fair value of the employee stock option grants were as follows:

	As of December 31,		
	2014	2013	2012
Risk-free interest rate	1.90%	1.62%	2.29%
Expected volatility	80%	79%	84%
Expected term (in years)	6.0	5.6	5.9
Expected dividend yield	0.0%	0.0%	0.0%

Risk-free interest rate. The Company bases the risk-free interest rate assumption on observed interest rates appropriate for the expected term of the stock option grants.

Expected volatility. The expected volatility assumption is based on volatilities of a peer group of similar companies whose share prices are publicly available. The peer group was developed based on companies in the biotechnology industry.

Expected term. The expected term represents the period of time that options are expected to be outstanding. Because the Company does not have historical exercise behavior, it determines the expected life assumption using the simplified method, which is an average of the contractual term of the option and its vesting period.

Expected dividend yield. The Company bases the expected dividend yield assumption on the fact that it has never paid cash dividends and has no present intention to pay cash dividends.

The allocation of stock-based compensation for all equity awards is as follows (in thousands):

	As of December 31,		
	2014	2013	2012
Research and development	$1,712	$1,264	$222
General and administrative	1,607	124	76
	$3,319	$1,388	$298

As of December 31, 2014 the unrecognized compensation cost related to outstanding employee options was $8.9 million and is expected to be recognized as expense over approximately 3.0 years.

Common Stock Reserved for Future Issuance

Common stock reserved for future issuance as of December 31, 2014 and 2013 is as follows:

	December 31,	
	2014	2013
Granted and outstanding under the Plans	2,408,634	1,543,667
Available for grant under the 2012 Plan	294,845	26,294
Available for issuance under Employee Stock Purchase Plan	140,711	—
Common stock warrants issued and outstanding	206,340	702
Convertible preferred stock warrants issued and outstanding	—	231,821
Convertible preferred stock	—	11,151,192
	3,050,530	12,953,676

8. Income Taxes

The following is a reconciliation of the expected statutory federal income tax provision to the actual income tax provision (in thousands):

	December 31,		
	2014	2013	2012
Tax computed at federal statutory rate	$(11,510)	$ (6,831)	$(5,396)
State income tax, net of federal benefit	(1,517)	(987)	(907)
Non-deductible interest	20	20	36
Other permanent items	1,676	756	214
Research credits	(557)	(728)	(93)
Remove (restore) DTA for NOL and Credits—IRC 382	—	(12,666)	2,135
State Taxes	—	—	—
Uncertain tax position	(1,125)	859	—
Valuation allowance	13,013	19,577	4,011
Provision (benefit) for income taxes	$ —	$ —	$ —

The components of the Company's deferred tax assets are summarized as follows (in thousands):

	December 31,	
	2014	2013
Deferred tax assets:		
Net operating loss carryforwards	$ 31,484	$ 19,288
Research credits	1,649	1,092
Capitalized R&D	5,410	6,391
Other	2,049	803
Deferred tax assets	40,592	27,574
Valuation allowance	(40,592)	(27,574)
Net deferred tax assets	$ —	$ —

The Company has established a valuation allowance for all deferred tax assets (DTA) including those for new operating loss and tax credit carryforwards. A valuation allowance of approximately $40.6 million of which approximately $13.0 million relates to 2014, has been recognized to offset the deferred tax assets, as realization of such assets is uncertain.

At December 31, 2014, the Company had federal and California net operating loss (NOL) carryforwards of approximately $82.2 million and $77.0 million, respectively. The federal NOL carryforwards will begin to expire in 2027 unless previously utilized, and the state NOL carryforwards have already begun to expire, and will continue to do so, unless utilized. At December 31, 2014, the Company had federal and state research tax credits each of $1.3 million. The federal research tax credits begin to expire in 2032 unless previously utilized. The California research credit will carry forward indefinitely until utilized.

Utilization of the NOL and research tax credit carryforwards may be subject to a substantial annual limitation due to ownership change limitations that have occurred or that could occur in the future, as required by Section 382 of the Code, as well as similar state and foreign provisions. These ownership changes may limit the amount of NOL and research tax credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. In general, an "ownership change" as defined by Section 382 of the Code results from a transaction or series of transactions over a three-year period resulting in an ownership change of more than 50 percentage points of the outstanding stock of a company by certain stockholders or public groups.

The Company completed a study to assess whether an ownership change, as defined by Section 382 of the Code, had occurred from the Company's formation through December 31, 2014. Based upon this study, the Company determined that several ownership changes had occurred. Accordingly, the Company has reduced its deferred tax assets related to the federal and state NOL carryforwards and the federal research tax credit carryforwards that are anticipated to expire unused as a result of these ownership changes. These tax attributes have been excluded from the deferred tax assets with a corresponding reduction in the valuation allowance with no net effect on income tax expense or the effective tax rate. Future ownership changes may further limit the Company's ability to utilize its remaining tax attributes.

The Company adopted the provisions of Financial Accounting Standards Board (FASB) ASC 740-10 *Income Taxes*, relating to accounting for uncertain tax positions on July 1, 2009.

The following table summarized the activity related to the Company's unrecognized tax benefits (in thousands):

	Year Ended December 31,	
	2014	2013
Balance beginning of the year	$ 1,709	$ —
Increase related to prior year tax positions	(1,361)	681
Increase related to current year tax positions	314	1,028
Balance at end of year	$ 662	$ 1,709

There were no unrecognized tax benefits prior to 2013. Approximately $0.7 million of the unrecognized tax benefits would reduce the Company's annual effective tax rate, if recognized, subject to the valuation allowance. It is not anticipated that there will be significant change in the unrecognized tax benefits over the next 12 months.

Due to the net operating loss carryforwards, the U.S. federal and state returns are open to examination by the Internal Revenue Service and significant state and foreign jurisdictions for all years beginning with the inception of the Company. The Company's policy is to recognize interest expense and penalties related to income tax matters as a component of income tax expense. There was no interest and penalties associated with uncertain tax positions as of December 31, 2014.

9. Employee Benefits

All employees of the Company are eligible to participate in the 401(k) Plan. The 401(k) matching contributions, if any, are determined by the Company at its sole discretion. During the years ended December 31, 2014, 2013 and 2012, the Company made matching contributions totaling $0.3 million, $0.1 million, $0.1 million, respectively.

10. Subsequent Events

Novasep Agreement

On March 20, 2015, the Company entered into a Development, Manufacturing and Supply Agreement (the "Manufacturing Agreement") with Novasep, Inc. ("Novasep") which superseded the Letter Agreement dated December 19, 2014 by and between the Company and Novasep. Under the terms of the Manufacturing Agreement, the parties agreed to continue the work initiated under the Letter Agreement, including the work necessary to prepare for the potential manufacture of MYDICAR drug substance (AAV1/SERCA2a) at the facilities of Novasep's affiliate Henogen in Europe (the "Novasep Facility"). Pursuant to the Manufacturing Agreement (and as previously agreed in the Letter Agreement), in exchange for payments from the Company to Novasep totaling up to €4,750,000, Novasep agreed to (i) conduct the engineering design work for facility modifications that would be necessary for the manufacture of MYDICAR drug substance, (ii) undertake initial process and analytical transfer and initial scale-up work in support of such potential future commercial manufacturing of MYDICAR drug substance, and (iii) allocate the resources and capacity necessary for the foregoing activities. The parties have also agreed to proceed with the additional process transfer, engineering/construction, scale-up and development activities necessary for future production of MYDICAR drug substance in accordance with current Good Manufacturing Practices ("GMP"), and agreed to terms of a commercial supply arrangement with a term through at least December 31, 2018, with extension options through 2020 in favor of the Company. The Company has the right to terminate the Manufacturing Agreement, exercisable for a specified period of time following the un-blinding of the data from the Company's Phase 2b clinical trial of MYDICAR (CUPID 2), if the Company concludes in good faith that the CUPID 2 data is such that the Company does not require production of MYDICAR drug substance at the Novasep Facility. The Company expects to un-blind the data from the CUPID 2 trial in late April 2015.

Unless the Company exercises the post CUPID 2 data termination right described above, the Company will be obligated to (i) fund Novasep's modifications to the Novasep Facility through time- and event-triggered milestone payments, (ii) make additional payments for the development services to be performed by Novasep, and (iii) commit to purchase a specified number of batches of MYDICAR drug substance (or make minimum payments with respect to any such batches that are not purchased) through 2018 (if the Company elects that the Novasep Facility be operated as a multi-product facility) or through 2019 (if the Company elects to have the Novasep Facility dedicated to MYDICAR drug substance production during the term of the Manufacturing Agreement).

In addition to the above-described post CUPID 2 data termination right, the Company has the right to terminate the Manufacturing Agreement (i) at will on or before March 31, 2016, (ii) following the shut-down or non-production of the Novasep Facility for a specified period of time, or (iii) upon Novasep's debarment. Additionally, each party may terminate the Manufacturing Agreement upon uncured material breach thereof by the other party, upon the other party's insolvency or bankruptcy, or in the event of a continuing force majeure preventing performance. Upon any termination of the Manufacturing Agreement by Celladon following the expiration of the post CUPID 2 data termination right either for convenience or for any reason other than material breach of the Manufacturing Agreement, shut-down or non-production of the Novasep Facility for a period extending longer than six months, or Novasep's insolvency, the Company is obligated to pay previously-unreimbursed amounts incurred by Novasep and specified termination fees as set forth in the Manufacturing Agreement.

11. Selected Quarterly Financial Data (Unaudited)

The following financial information reflects all normal recurring adjustments, which are, in the opinion of management, necessary for a fair statement of the results of the interim periods. Summarized quarterly data for fiscal 2014 and 2013 are as follows (in thousands, except per share data):

	First Quarter	Second Quarter	Third Quarter	Fourth Quarter
2014				
Total operating expenses	$ 6,924	$ 7,005	$ 8,131	$ 10,958
Consolidated Net loss	(7,162)	(6,992)	(8,358)	(11,341)
Basic and diluted net loss per share	$ (0.60)	$ (0.38)	$ (0.40)	$ (0.49)
2013				
Total operating expenses	$ 3,472	$ 4,992	$ 5,523	$ 5,977
Consolidated Net loss	(3,530)	(4,929)	(5,453)	(6,179)
Basic and diluted net loss per share	$ (3.99)	$ (8.98)	$ (6.17)	$ (7.96)

ITEM 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

ITEM 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures.

We are responsible for maintaining disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Disclosure controls and procedures are controls and other procedures designed to ensure that the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and our principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

Based on our management's evaluation (with the participation of our principal executive officer and our principal financial officer) of our disclosure controls and procedures as required by Rule 13a-15 under the Exchange Act, our principal executive officer and our principal financial officer have concluded that our disclosure controls and procedures were effective to achieve their stated purpose as of December 31, 2014, the end of the period covered by this report.

Management's Report on Internal Control over Financial Reporting.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as such term is defined in Exchange Act Rule 13a-15(f). Internal control over financial reporting is a process designed under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States of America.

As of December 31, 2014, our management assessed the effectiveness of our internal control over financial reporting using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in Internal Control-Integrated Framework (2013 Framework). Based on this assessment, our management concluded that, as of December 31, 2014, our internal control over financial reporting was effective based on those criteria.

Changes in Internal Control over Financial Reporting.

There were no changes in our internal control over financial reporting during the quarter ended December 31, 2014 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. Other Information

None.

PART III

ITEM 10. Directors, Executive Officers and Corporate Governance

The information required by this item and not set forth below will be set forth in the sections entitled “Election of Directors,” “Executive Officers” and “Section 16(a) Beneficial Ownership Reporting Compliance” in our definitive proxy statement for our 2015 Annual Meeting of Stockholders, or our Proxy Statement, to be filed with the SEC within 120 days after the end of the fiscal year ended December 31, 2014, and is incorporated herein by reference.

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the code is posted on the Investors Corporate Governance section of our website, which is located at www.celladon.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

ITEM 11. Executive Compensation

The information required by this item will be set forth in the section entitled “Executive Compensation” in our Proxy Statement and is incorporated herein by reference.

ITEM 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item will be set forth in the sections entitled “Equity Compensation Plan Information” and “Principal Stockholders” in our Proxy Statement and is incorporated herein by reference.

ITEM 13. Certain Relationships and Related Party Transactions, and Director Independence

The information required by this item will be set forth in the sections entitled “Certain Relationships and Related Party Transactions” and “Election of Directors” in our Proxy Statement and is incorporated herein by reference.

ITEM 14. Principal Accounting Fees and Services

The information required by this item will be set forth in the section entitled “Principal Accounting Fees and Services” in our Proxy Statement and is incorporated herein by reference.

PART IV

ITEM 15. Exhibits and Financial Statement Schedules

(a) Financial Statements and Financial Statement Schedules

1. Financial Statements:

The financial statements of Celladon Corporation listed below are set forth in Item 8 of this Report for the year ended December 31, 2014:

2. Financial Statement Schedules:

All other schedules are omitted because they are not applicable or the required information is shown in the financial statements or notes thereto.

(b) Exhibits

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on February 10, 2014).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on February 10, 2014).
4.1	Reference is made to Exhibits 3.1 and 3.2.
4.2	Form of Common Stock Certificate of the Registrant (incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
4.3	Amended and Restated Investor Rights Agreement by and among the Registrant and certain of its stockholders, dated February 4, 2014 (incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
4.4	Form of Warrant to Purchase Common Stock issued to participants in the Registrant's Convertible Debt and Warrant financing, dated October 15, 2013 (incorporated by reference to Exhibit 4.3 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.1+	Form of Indemnity Agreement by and between the Registrant and its directors and officer (incorporated by reference to Exhibit 10.1 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.2+	Celladon Corporation 2001 Stock Option Plan and Form of Notice of Grant of Stock Option, Stock Option Agreement and Stock Option Exercise Notice thereunder (incorporated by reference to Exhibit 10.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).

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10.3+	Celladon Corporation 2012 Equity Incentive Plan and Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise thereunder (incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
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10.6+	Celladon Corporation Non-Employee Director Compensation Policy (incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.7+	Employment Agreement by and between the Registrant and Jeffrey J. Rudy, dated September 3, 2013, as amended (incorporated by reference to Exhibit 10.7 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.8+	Employment Agreement by and between the Registrant and Rebecque Laba, dated September 3, 2013, as amended (incorporated by reference to Exhibit 10.8 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.9+	Employment Agreement by and between the Registrant and Ryan K. Takeya, dated September 2, 2013, as amended (incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.10+	Employment Agreement by and between the Registrant and Fredrik Wiklund, dated September 3, 2013, as amended (incorporated by reference to Exhibit 10.10 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.11+	Employment Agreement by and between the Registrant and Krisztina M. Zsebo, Ph.D., dated August 30, 2013, as amended (incorporated by reference to Exhibit 10.11 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.12+	Letter Agreement by and between the Registrant and Gregg Huber Alton, dated August 30, 2013 (incorporated by reference to Exhibit 10.12 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.13+	Letter Agreement by and between the Registrant and Graham Cooper, dated September 2, 2013 (incorporated by reference to Exhibit 10.13 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.14	Office Lease by and between the Registrant and Arden Realty, Inc., dated March 6, 2012, as amended (incorporated by reference to Exhibit 10.14 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.15*	License Agreement by and between the Registrant and the Regents of the University of California, dated February 10, 2001, as amended (incorporated by reference to Exhibit 10.15 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).

Exhibit Number	Description
10.16*	Exclusive License Agreement by and between the Registrant and Martin J. Kaplitt, M.D., dated June 7, 2006 (incorporated by reference to Exhibit 10.16 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.17*	Non-Exclusive License Agreement by and between the Registrant and AskBio, LLC, dated January 15, 2008 (incorporated by reference to Exhibit 10.17 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.18	License Agreement by and between the Registrant and AdVec Inc., dated February 24, 2009 (incorporated by reference to Exhibit 10.18 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.19*	Exclusive Patent License Agreement by and between the Registrant and the Regents of the University of Minnesota, dated May 11, 2009 (incorporated by reference to Exhibit 10.19 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.20*	Non-Exclusive License Agreement by and between the Registrant and Virovek Incorporation, dated November 4, 2010 (incorporated by reference to Exhibit 10.20 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.21*	Amended and Restated License Agreement by and between the Registrant and AmpliPhi Biosciences Corporation, dated June 27, 2012 (incorporated by reference to Exhibit 10.21 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.22*	Sublicense Agreement by and between the Registrant and AmpliPhi Biosciences Corporation, dated June 27, 2012 (incorporated by reference to Exhibit 10.22 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.23*	Amended and Restated Manufacturing Services Agreement by and between the Registrant and Lonza Houston, Inc., dated August 26, 2013 (incorporated by reference to Exhibit 10.23 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.24+	Letter Agreement by and between the Registrant and Michael Narachi, dated October 16, 2013 (incorporated by reference to Exhibit 10.24 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.25*	Material Transfer and Exclusivity Agreement by and between the Registrant and Les Laboratoires Servier, dated February 20, 2014 (incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q filed with the SEC on May 13, 2014).
10.26	Sublease Agreement by and between the Registrant and Brandes Investment Partners, L.P. dated May 28, 2014 (incorporated by reference to Exhibit 10.26 to the Registrant's Registration Statement on Form S-1 (File No. 333-197720), originally filed with the SEC on July 30, 2014).
10.27	Assignment and License Agreement by and between the Registrant and Enterprise Management Partners, LLC dated July 18, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on July 21, 2014).
10.28+	Employment Agreement by and between the Registrant and Paul Cleveland, dated May 28, 2014 (incorporated by reference to Exhibit 10.28 to the Registrant's Registration Statement on Form S-1 (File No. 333-197720), originally filed with the SEC on July 30, 2014).

Exhibit Number	Description
10.29+	Employment Agreement by and between the Registrant and Elizabeth Reed, dated May 30, 2014 (incorporated by reference to Exhibit 10.29 to the Registrant's Registration Statement on Form S-1 (File No. 333-197720), originally filed with the SEC on July 30, 2014).
10.30	Loan and Security Agreement by and between the Registrant, Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc., dated as of July 31, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on August 5, 2014).
10.31	Assignment and License Agreement by and between the Registrant and Enterprise Management Partners, LLC dated July 18, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on July 21, 2014).
10.32*	Letter Agreement by and between the Registrant and Novasep, Inc, dated as of December 19, 2014.
10.33	Amendment No. 1 to Loan and Security Agreement, dated December 10, 2014, by and between the Registrant, Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc.
10.34*	Facility Construction and Commercial Supply Agreement, dated as of October 31, 2014, by and between the Registrant and Lonza Biologics, Inc.
10.35*	Development, Manufacturing and Supply Agreement, dated March 20, 2015, by and between the Registrant and Novasep, Inc.
23.1	Consent of Independent Registered Public Accounting Firm.
24.1	Power of Attorney. Reference is made to the signature page hereto.
31.1	Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
31.2	Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
32.1	Certification of the Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certification of the Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

+ Indicates management contract or compensatory plan.

* Confidential treatment has been requested or granted with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Celladon Corporation

Date: March 31, 2015

By: /s/ Paul B. Cleveland
Paul B. Cleveland
President and Chief Financial Officer

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Krisztina M. Zsebo and Paul B. Cleveland, and each of them, as his or her attorneys-in-fact, with the power of substitution, for him or her in any and all capacities, to sign any amendments to this report, and to file the same, with exhibits thereto and other documents in connection therewith with the Securities and Exchange Commission, hereby ratifying and confirming all that said attorneys-in-fact, and each of them, or his or her substitute or substitutes may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Krisztina M. Zsebo Krisztina M. Zsebo, Ph.D.	Chief Executive Officer and Member of the Board of Directors *(Principal Executive Officer)*	March 31, 2015
/s/ Paul B. Cleveland Paul B. Cleveland	President and Chief Financial Officer *(Principal Financial Officer)*	March 31, 2015
/s/ Andrew C. Jackson Andrew C. Jackson	Corporate Controller *(Principal Accounting Officer)*	March 31, 2015
/s/ Michael Narachi Michael Narachi	Chairman of the Board of Directors	March 31, 2015
/s/ Gregg Alton Gregg Alton	Member of the Board of Directors	March 31, 2015
/s/ Graham Cooper Graham Cooper	Member of the Board of Directors	March 31, 2015
/s/ Joshua Funder Joshua Funder, Ph.D.	Member of the Board of Directors	March 31, 2015
/s/ Peter K. Honig Peter K. Honig, M.D., M.P.H	Member of the Board of Directors	March 31, 2015
/s/ Patrick Y. Yang Patrick Y. Yang, Ph.D.	Member of the Board of Directors	March 31, 2015

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INDEX TO EXHIBITS

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10.23*	Amended and Restated Manufacturing Services Agreement by and between the Registrant and Lonza Houston, Inc., dated August 26, 2013 (incorporated by reference to Exhibit 10.23 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.24+	Letter Agreement by and between the Registrant and Michael Narachi, dated October 16, 2013 (incorporated by reference to Exhibit 10.24 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-191688), originally filed with the SEC on October 11, 2013).
10.25*	Material Transfer and Exclusivity Agreement by and between the Registrant and Les Laboratoires Servier, dated February 20, 2014 (incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q filed with the SEC on May 13, 2014).
10.26	Sublease Agreement by and between the Registrant and Brandes Investment Partners, L.P. dated May 28, 2014 (incorporated by reference to Exhibit 10.26 to the Registrant's Registration Statement on Form S-1 (File No. 333-197720), originally filed with the SEC on July 30, 2014).
10.27	Assignment and License Agreement by and between the Registrant and Enterprise Management Partners, LLC dated July 18, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on July 21, 2014).
10.28+	Employment Agreement by and between the Registrant and Paul Cleveland, dated May 28, 2014 (incorporated by reference to Exhibit 10.28 to the Registrant's Registration Statement on Form S-1 (File No. 333-197720), originally filed with the SEC on July 30, 2014).
10.29+	Employment Agreement by and between the Registrant and Elizabeth Reed, dated May 30, 2014 (incorporated by reference to Exhibit 10.29 to the Registrant's Registration Statement on Form S-1 (File No. 333-197720), originally filed with the SEC on July 30, 2014).
10.30	Loan and Security Agreement by and between the Registrant, Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc., dated as of July 31, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on August 5, 2014).
10.31	Assignment and License Agreement by and between the Registrant and Enterprise Management Partners, LLC dated July 18, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on July 21, 2014).
10.32*	Letter Agreement by and between the Registrant and Novasep, Inc, dated as of December 19, 2014.
10.33	Amendment No. 1 to Loan and Security Agreement, dated December 10, 2014, by and between the Registrant, Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc.
10.34*	Facility Construction and Commercial Supply Agreement, dated as of October 31, 2014, by and between the Registrant and Lonza Biologics, Inc.
10.35*	Development, Manufacturing and Supply Agreement, dated March 20, 2015, by and between the Registrant and Novasep, Inc.
23.1	Consent of Independent Registered Public Accounting Firm.
24.1	Power of Attorney. Reference is made to the signature page hereto.
31.1	Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.

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Exhibit Number	Description
31.2	Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
32.1	Certification of the Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certification of the Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

+ Indicates management contract or compensatory plan.
* Confidential treatment has been requested or granted with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.

Exhibit 10.32

*****Text Omitted and Filed Separately with the Securities and Exchange Commission. Confidential Treatment Requested Under 17 C.F.R. Sections 200.80(b)(4) and 240.24b-2.**

December 19, 2014

Novasep, Inc.
23 Creek Circle
Boothwyn, PA 19061
USA

Re: Mydicar Commercial Supply Project

Ladies and Gentlemen:

As we have discussed, Celladon Corporation, a Delaware corporation (***"Celladon"***), and Novasep, Inc., a New Jersey corporation (***"Novasep"***), are in discussions regarding a potential collaborative relationship related to Celladon's proprietary AAV1/SERCA2a gene therapy candidate known as MYDICAR®, pursuant to which the parties would implement a transfer program to enable Novasep to produce Mydicar on behalf of Celladon, Novasep would make facility modifications necessary for the manufacture of Mydicar and perform process development, scale-up and validation services necessary for commercial production of Mydicar, and Novasep would manufacture and supply Mydicar to Celladon for commercial distribution (collectively, the ***"Project"***). For the sake of clarity, fill and finish services or activities are excluded from Novasep's scope of services and supply.

The purpose of this letter agreement (the ***"Letter"***) is: (i) to set forth the parties' binding rights and obligations with respect to certain preliminary Project activities that the parties wish to commence, or reserve capacity for (as applicable), prior to the negotiation and execution by the parties of a definitive agreement governing the Project (a ***"Definitive Agreement"***); and (ii) to facilitate the discussion and negotiation of a Definitive Agreement.

The parties, intending to be legally bound (except with respect to **Schedule B** to this Letter, which is not binding on either party), agree as follows:

1. Initial Activities. Novasep (itself and/or through its affiliate Novasep Belgium) agrees: (a) to perform the activities described in **Schedule A** to this Letter (the ***"Initial Project Plan"***) in accordance with the terms to be negotiated in good faith by the Parties and in compliance with applicable laws, rules and regulations; and (b) to use commercially reasonable efforts to perform such activities and deliver the deliverables to be agreed to by the Parties substantially in accordance with the timelines to be agreed.

2. Payments. Celladon agrees to compensate Novasep for the performance of the Initial Project Plan in an aggregate amount of up to four million seven hundred fifty thousand Euro (4,750,000 €), which shall be payable as follows:

(a) one million Euro (1,000,000 €) within three business days after the date of this Letter;

(b) one million five hundred thousand Euro (1,500,000 €) no later than […***…];

(c) six hundred thousand Euro (600,000 €) no later than […***…];

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(d) eight hundred twenty five thousand Euro (825 000 €) no later than […***…]; and

(e) eight hundred twenty five thousand Euro (825 000 €) no later than […***…].

3. Definitive Agreement. Commencing as promptly as practicable after the date of this Letter, the parties shall negotiate in good faith a Definitive Agreement on commercially reasonable terms. Upon signature by the parties, the Definitive Agreement would supersede this Letter. **Exhibit B** to this Letter outlines some of the key principles and terms that the parties expect to include in a Definitive Agreement. However, the parties acknowledge that **Exhibit B** hereto is a statement of intent only, is intended to facilitate the negotiation of a Definitive Agreement, is not intended to be, and is not, legally binding on either party, and addresses only a limited subset of the terms that would need to be included in a Definitive Agreement. The parties further acknowledge that the key principles and terms outlined in **Exhibit B** will require further discussion, elaboration and qualification and may require modification. The parties will use commercially reasonable efforts to conclude a Definitive Agreement as promptly as practicable and in any event by March 31, 2015; *provided, however,* that if Celladon exercises its termination right under Section 4(a) or Section 4(b) of this Letter prior to signature by the parties of a Definitive Agreement, then the parties shall have no further obligation to negotiate a Definitive Agreement.

4. […***…]:

[…***…]

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[...***...]

5. Confidentiality. All information and materials disclosed or transferred by Celladon to Novasep pursuant to the Initial Project Plan shall at all times be owned solely by Celladon and shall be considered "Confidential Information" of Celladon for purposes of that certain Mutual Confidential Disclosure Agreement between Celladon and Groupe Novasep SAS dated August 15, 2011 (the ***"Non-Disclosure Agreement"***), which shall remain in full force and effect in accordance with its terms; *provided, however,* that Novasep shall have the right to use the information and materials disclosed or transferred by Celladon to Novasep pursuant to the Initial Project Plan for the purpose of performing the Initial Project Plan (but not for any other purpose); and *provided, further,* that, (a) notwithstanding Section 7 of the Non-Disclosure Agreement to the contrary, the expiration date of the Non-Disclosure Agreement is hereby extended to December 19, 2024, and (b) with respect to any Confidential Information of Celladon that is specifically identified to be a trade secret under applicable law, Novasep's obligations under the Non-Disclosure Agreement shall survive the expiration thereof for so long as such Confidential Information remains a trade secret.

6. Intellectual Property. Celladon shall solely own, and Novasep hereby assigns to Celladon, all right, title and interest in and to any invention, development or discovery (whether or not patentable) made or generated by or on behalf of Novasep or Novasep Belgium in the course of performance of the Initial Project Plan that constitutes a modification or improvement of, or that uses or incorporates, Celladon's Confidential Information (as defined in the Non-Disclosure Agreement and supplemented by Section 5 of this Letter), including all intellectual property rights therein. Celladon shall grant a non-exclusive, royalty-free license to Novasep, Novasep Belgium, and their affiliates to use any invention, development or discovery (whether or not patentable) made or generated by or on behalf of Novasep or Novasep Belgium in the course of performance of the Initial Project Plan that is generally applicable to the development or manufacture of biological products or product components and does not use, require the use of, or incorporate any of Celladon's Confidential Information.

7. Survival. The parties' respective rights and obligations under Sections 4, 5, 6, 7 and 8 of this Letter and under the Non-Disclosure Agreement (as supplemented by this Letter) shall survive termination of this Letter or the Definitive Agreement (as applicable) pursuant to Section 4 of this Letter.

8. Miscellaneous. Each party will be responsible for and bear all of its own costs and expenses incurred in connection with the negotiation of this Letter and the Definitive Agreement. Novasep's relationship with Celladon is that of an independent contractor and nothing in this Letter should be construed to create a partnership, joint venture, or employer-employee relationship. This Letter shall be governed by the laws of the State of New Jersey, USA, excluding its conflicts of laws principles. This Letter, including the Exhibits hereto, together with the Non-Disclosure Agreement, constitutes the complete and exclusive agreement of the parties with respect to the subject matter hereof and thereof. This Letter may not be modified except by

a written instrument signed by both parties. No failure or delay of one of the parties to insist upon strict performance of any of its rights or powers under this Letter shall operate as a waiver thereof, nor shall any other single or partial exercise of such right or power preclude any other further exercise of any rights or remedies provided by law. This Letter may be executed in multiple counterparts (including by facsimile), each of which shall be deemed an original and all of which together shall constitute one and the same instrument.

If this Letter is acceptable to you, please indicate your acceptance by countersigning this Letter below and returning a copy to me.

Sincerely,

CELLADON CORPORATION

By: /s/ Paul B. Cleveland
Paul B. Cleveland
President and Chief Financial Officer

Agreed to and accepted as of the date first set forth above:

NOVASEP, INC.

By: /s/ Andrew Brennan

Name: Andrew Brennan
Title: General Manager, US Operations

Schedule A

Initial Project Plan

[…***…]

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Exhibit B

Non-Binding Summary of Terms

[…***…]

*****Confidential Treatment Requested**

AMENDMENT NO. 1
TO
LOAN AND SECURITY AGREEMENT

THIS AMENDMENT NO. 1 TO LOAN AND SECURITY AGREEMENT (this "Amendment") is dated as of December 10, 2014 (the "First Amendment Date") and is entered into by and among CELLADON CORPORATION, a Delaware corporation, and each of its subsidiaries (hereinafter collectively referred to as the "Borrower"), HERCULES TECHNOLOGY III, L.P., a Delaware limited partnership (HT III"), and HERCULES TECHNOLOGY GROWTH CAPITAL, INC., a Maryland corporation ("HTGC"). Capitalized terms used herein without definition shall have the same meanings given them in the Loan Agreement (as defined below).

RECITALS

A. Borrower, Agent and Lender have entered into that certain Loan and Security Agreement dated as of July 31, 2014 (as may be amended, restated, or otherwise modified, the "Loan Agreement"), pursuant to which Lender has agreed to extend and make available to Borrower certain advances of money.

B. Borrower, Agent and Lender desire to amend the Loan Agreement upon the terms and conditions more fully set forth herein.

AGREEMENT

NOW, THEREFORE, in consideration of the foregoing Recitals and intending to be legally bound, the parties hereto agree as follows:

1. AMENDMENTS.

1.1 The definition of "Funding Milestone" in the Loan Agreement is hereby amended and restated in its entirety as follows:

"Funding Milestone" means the delivery of a written notice from the Borrower to Agent that states that the Phase2b data supports the continued development of MYDICAR for reducing hospitalization for heart failure in Nab-negative NYHA class III or class IV heart failure patients who are not in immediate need of a left ventricular assist device or heart transplant, with the next clinical step for such indication (other than the two previously planned studies as disclosed to Agent) being a phase III study or for registration for approval, all as reasonably determined by Borrower's senior management and board of directors not later than June 30, 2015. For the avoidance of doubt, any determination by the Borrower's senior management and board of directors that the Phase2b data supports the development of MYDICAR for other indications or supports the conduct of one or more different studies at any phase level will not prevent the occurrence of a Funding Milestone that would otherwise be deemed to have occurred upon satisfying the conditions in the previous sentence.

1.2 Section 2.1(a)(ii). Section 2.1(a)(ii) of the Loan Agreement is hereby amended and restated in its entirety as follows:

(ii) Subject to the terms and conditions of this Agreement, beginning on the date that the Funding Milestone has been achieved and continuing through June 30, 2015, Lender will severally (and not jointly) make in an amount not to exceed its respective Term Commitment, the Second Tranche Advance.

2. BORROWER'S REPRESENTATIONS AND WARRANTIES. Borrower represents and warrants to Lender and Agent that:

2.1 Immediately upon giving effect to this Amendment (i) the representations and warranties contained in the Loan Documents are true, accurate and complete in all material respects as of the date hereof (except to the extent such representations and warranties relate to an earlier date, in which case they are true and correct as of such date), and (ii) no Event of Default has occurred and is continuing with respect to which Borrower has not been notified in writing by Agent or Lender.

2.2 Borrower has the corporate power and authority to execute and deliver this Amendment and to perform its obligations under the Loan Agreement, as amended by this Amendment.

2.3 The certificate of incorporation, bylaws and other organizational documents of Borrower delivered to Agent and Lender on the Closing Date remain true, accurate and complete and have not been amended, supplemented or restated and are and continue to be in full force and effect.

2.4 The execution and delivery by Borrower of this Amendment and the performance by Borrower of its obligations under the Loan Agreement, as amended by this Amendment, have been duly authorized by all necessary corporate action on the part of Borrower.

2.5 This Amendment has been duly executed and delivered by Borrower and is the binding obligation of Borrower, enforceable against it in accordance with its terms, except as such enforceability may be limited by bankruptcy, insolvency, reorganization, liquidation, moratorium or other similar laws of general application and equitable principles relating to or affecting creditors' rights; and

2.6 As of the date hereof, it has no defenses against the obligations to pay any amounts under the Obligations. Borrower acknowledges that Lender and Agent have acted in good faith and have conducted in a commercially reasonable manner their relationships with Borrower in connection with this Amendment.

Borrower understands and acknowledges that Lender and Agent are entering into this Amendment in reliance upon, and in partial consideration for, the above representations and warranties, and agrees that such reliance is reasonable and appropriate.

3. LIMITATION. The amendments set forth in this Amendment shall be limited precisely as written and shall not be deemed (a) to be a waiver or modification of any other term or condition of the Loan Agreement or of any other instrument or agreement referred to therein or to prejudice any right or remedy which Agent or Lender may now have or may have in the future under or in connection with the Loan Agreement (as amended hereby) or any instrument or agreement referred to therein; or (b) to be a consent to any future amendment or modification or waiver to any instrument or agreement the execution and delivery of which is consented to hereby, or to any waiver of any of the provisions thereof. Except as expressly amended hereby, the Loan Agreement shall continue in full force and effect.

4. RESERVED

5. EFFECTIVENESS. This Amendment shall become effective upon the satisfaction of all the following condition precedent:

5.1 Amendment. Borrower, Agent and Lender shall have duly executed and delivered this Amendment to each other.

5.2 Payment of Lender Expenses. Borrower shall have paid all Lender expenses (including all reasonable attorneys' fees and reasonable expenses) incurred through the date of this Amendment.

6. COUNTERPARTS. This Amendment may be signed in any number of counterparts, and by different parties hereto in separate counterparts, with the same effect as if the signatures to each such counterpart were upon a single instrument. All counterparts shall be deemed an original of this Amendment. This Amendment may be executed by facsimile, portable document format (.pdf) or similar technology signature, and such signature shall constitute an original for all purposes.

7. INCORPORATION BY REFERENCE. The provisions of Section 11 of the Agreement shall be deemed incorporated herein by reference, *mutatis mutandis*.

8. SECOND TRANCHE ADVANCE REFINANCED. For the avoidance of doubt, the $15,000,000 commitment for the Second Tranche Advance set forth in Section 2.1(a)(ii) of the Loan Agreement in effect prior to this Amendment has been terminated in full and is now replaced by the Second Tranche Advance commitment set forth in this Amendment.

[SIGNATURES CONTINUED ON THE FOLLOWING PAGE]

IN WITNESS WHEREOF, the parties have duly authorized and caused this Amendment to be executed as of the date first written above.

BORROWER:

CELLADON CORPORATION

Signature: /s/ Paul B. Cleveland

Print Name: Paul B. Cleveland

Title: President and CFO

Accepted in Palo Alto, California:

AGENT AND LENDER:

HERCULES TECHNOLOGY GROWTH CAPITAL, INC.

Signature: /s/ Ben Bang
Name: Ben Bang
Title: Associate General Counsel

LENDER:

HERCULES TECHNOLOGY III, L.P.,
a Delaware limited partnership

By: Hercules Technology SBIC Management, LLC, its General Partner

By: Hercules Technology Growth Capital, Inc., its Manager

By: /s/ Ben Bang
Name: Ben Bang
Title: Associate General Counsel

Exhibit 10.34

*****Text Omitted and Filed Separately with the Securities and Exchange Commission. Confidential Treatment Requested Under 17 C.F.R. Sections 200.80(b)(4) and 240.24b-2.**

CONFIDENTIAL

Facility Construction and Commercial Supply Agreement

(the "Agreement")

by and between

Lonza Biologics, Inc.
101 International Drive
Portsmouth, NH 03801
USA

- hereinafter "Lonza" -

and

Celladon Corporation
11988 El Camino Real, Suite 650
San Diego, CA 92130-3579
USA

- hereinafter "Customer" -

Effective as of October 31, 2014 (the "Effective Date")

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Appendix G	Form of Common Stock Purchase Agreement
Appendix H	Customer Background Intellectual Property

Recitals

WHEREAS, Customer is engaged in the development and research of a viral therapeutic Product and requires assistance in the development and manufacture of such Product;

WHEREAS, Lonza and its Affiliates have expertise in the evaluation, development and manufacture of biologics, including viral therapeutics;

WHEREAS, Customer and Lonza's Affiliate, Lonza Houston, Inc., entered into that certain Amended and Restated Manufacturing Services Agreement dated August 26, 2013 ("Clinical Agreement") relating to the manufacture of Product for clinical use; and

WHEREAS, Customer wishes to engage Lonza for Services relating to the manufacture of the Product for further clinical use and for commercial use, as more fully described herein; and

WHEREAS, Lonza, or its Affiliate, is prepared to perform such Services for Customer on the terms and subject to the conditions set out herein.

NOW, THEREFORE, in consideration of the mutual promises contained herein, and for other good and valuable consideration, the parties intending to be legally bound, agree as follows:

1 Definitions and Interpretation

"Affiliate"	means any company, partnership or other entity which directly or indirectly Controls, is Controlled by or is under common Control with the relevant Party. "Control" means the ownership of more than fifty percent (50%) of the issued share capital or the legal power to direct or cause the direction of the general management and policies of the relevant Party.
"Agreement"	means this agreement incorporating all Appendices, as amended from time to time by written agreement of the Parties.
"Applicable Laws"	means all relevant U.S. and European Union federal, state and local laws, statutes, rules, and regulations which are applicable to a Party's activities hereunder, including, without limitation, the applicable regulations and guidelines of any Governmental Authority and all applicable cGMP together with amendments thereto.
"Approval"	means the approval by the FDA or EMA to market and sell Product manufactured at the Facility in the United States or the European Union, respectively.
	"Background Intellectual Property" means any Intellectual Property either (i) owned or controlled by a Party prior to the Effective Date or (ii) developed or acquired by a Party independently from the performance of the

	Services hereunder during the Term of this Agreement. Customer's Background Intellectual Property existing as of the Effective Date to be provided to Lonza under this Agreement is set forth in Appendix H.
"Batch"	means the Product derived from a single run of the Manufacturing Process at a 2000 L working volume.
"Building"	means the existing building located at […***…], Portsmouth, NH or such other building as may be agreed by the Parties.
"CapEx" or "CapEx Amount"	means the estimate of the capital cost expected to be incurred by Lonza to construct the Facility, as updated by Lonza […***…], excluding the […***…] and the […***…], which costs will be […***…].
"Capital Equipment"	means those certain pieces of equipment described in the Detailed Design or a Project Plan used to produce the Product that are purchased by Customer or for which Customer reimburses Lonza, including, without limitation, the related documentation regarding the design, validation, operation, calibration and maintenance of such equipment.
"Certificate of Analysis"	means a document prepared by Lonza listing tests performed by Lonza or approved External Laboratories, the Specifications and test results with respect to a Batch and such other information and certifications as are required to be in such document pursuant to the Quality Agreement.
"cGMP"	means those laws and regulations applicable in the U.S. and Europe, relating to the manufacture of medicinal products for human use, including, without limitation, current good manufacturing practices as specified in the ICH guidelines, including without limitation, ICH Q7A "ICH Good Manufacturing Practice Guide for Active Pharmaceutical Ingredients", US Federal Food Drug and Cosmetic Act at 21CFR (Chapters 210, 211, 600 and 610) and the Guide to Good Manufacturing Practices for Medicinal Products as promulgated under European Directive 91/356/EEC. For the avoidance of doubt, Lonza's operational quality standards are defined in internal cGMP policy documents.

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"cGMP Batch"	means a Batch that is required under the applicable Project Plan or Purchase Order to be manufactured in accordance with cGMP.
"Change"	means any change to the Services or pricing incorporated into a written amendment to the Agreement in accordance with Clause 16.3 or effected in accordance with the Quality Agreement.
"Commencement Date"	means the date of removal of the vial of cells from frozen storage for the production of a Batch.
"Conceptual Study"	means the study prepared by Lonza pursuant to a statement of work under the Clinical Agreement executed April 24, 2014.
"Confidential Information"	means Customer Information and/or Lonza Information, as the context requires.
"Construction Trigger"	has the meaning set forth in Clause 2.6.
"Customer Information"	means all information that is proprietary to Customer or an Affiliate of Customer or that is maintained in confidence by Customer or any Affiliate of Customer and that is disclosed by Customer or any Affiliate of Customer to Lonza under or in connection with this Agreement, including, without limitation, any and all Customer business plans, know-how and trade secrets, and any materials supplied by Customer to Lonza in accordance with any Project Plan.
"Customer Materials"	means any Raw Materials, components of Product, or other materials of any nature provided by Customer.
"Customer Withdrawal"	means a good faith determination by the Customer's board of directors, as a result of regulatory, safety and/or efficacy concerns regarding the Product, to cease and refrain from development and promotion of the Product and not to seek marketing approvals therefor, followed by a public announcement by Customer's management that Customer has decided to permanently cease and refrain from developing and promoting the Product.
"Dedicated Production Line"	means a production line consisting of all rooms used for Product manufacturing that are personnel accessed from the supply or return corridor of the Facility that, during the term of this Agreement, unless otherwise agreed, will

	be used only for Services provided under this Agreement.
"Deliver" or "Delivery"	means availability of Product for pick up by the applicable carrier at the Facility for shipment to Customer.
"Delivery Date"	means, with respect to any cGMP Batch, the date of Delivery of such cGMP Batch.
"Detailed Design"	means the detailed design for the Facility which Lonza has commissioned pursuant to a Project Plan hereunder.
"Downstream Materials"	means chromatography resins and media, anion exchange filter membranes, and UF membranes intended to refine or purify the Product, as specified in the Master Batch Record.
"Downstream Material Handling Fees"	means a fee payable on each order of Downstream Materials equal to […***…] percent ([…***…]%) of the acquisition cost of such Downstream Materials by Lonza that is charged to the Customer in addition to the cost of such Downstream Materials.
"EMA"	means the European Medicines Agency, or any successor agency thereto.
"Engineering Batch"	means a Batch that is intended to demonstrate the transfer of the Manufacturing Process to the Dedicated Production Line and the Facility.
"Engineering Run Phase"	means the period following Facility OQ, during which Lonza will complete Engineering Runs, and which period will end when […***…] ([…***…]) Engineering Batches have been successfully manufactured according to Specifications at the Facility, unless such period is deemed completed earlier in accordance with Clause 3.4.
"Escrow Account"	means an account with Escrow Agent, intended to secure payment of […***…], and to facilitate […***…], in accordance with the Escrow Agreement.
"Escrow Agent"	means […***…] or such other reputable U.S. financial institution offering escrow services as may be mutually agreed by the Parties.

"Escrow Agreement"	means a three-party escrow agreement among Customer, Lonza and Escrow Agent in substantially the form attached as Appendix E, which will […***…].
"External Laboratories"	means any Third Party instructed by Lonza, with Customer's prior consent, which is to conduct activities required to complete the Services.
"Facility"	means a commercial scale viral production facility to be constructed by Lonza in the Building or such other Lonza facility as may be agreed upon by the Parties.
"Facility Mechanical Completion"	means that all equipment and mechanical systems necessary to produce up to […***…] Batches per year at the Facility have been installed but have not been commissioned.
"Facility OQ"	means all equipment and mechanical systems necessary to produce up to […***…] Batches per year at the Facility have been commissioned and operationally qualified.
"FDA"	means the United States Food and Drug Administration, or any successor agency thereto.
"First Approval"	means the first Approval of Product in the U.S. or Europe.
"Governmental Authority"	means any Regulatory Authority and any national, multi-national, regional, state or local regulatory agency, department, bureau, or other governmental entity in the U.S. or European Union.
"Intellectual Property"	means (i) inventions (whether or not patentable), patents, trade secrets, copyrights, trademarks, trade names and domain names, rights in designs, rights in computer software, database rights, rights in confidential information (including know-how) and any other intellectual property rights, in each case whether registered or unregistered, (ii) all applications (or rights to apply) for, and renewals or extensions of, any of the rights described in the foregoing clause (i), and (iii) and all rights and applications that are similar or equivalent to the rights and application described in the foregoing clauses (i) and (ii), which exist now, or which come to exist in the future, in any part of the world.

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"Lonza Information"	means all information that is proprietary to Lonza or any Affiliate of Lonza or that is maintained in confidence by Lonza or any Affiliate of Lonza and that is disclosed by Lonza or any Affiliate of Lonza to Customer under or in connection with this Agreement, including without limitation, any and all Lonza business plans, know-how and trade secrets.
"Lonza Responsibility"	means, in the case of any non-conformity of a Batch to the Product Warranty, that such non-conformity: (a) is primarily attributable to Lonza's breach of its obligations hereunder or Lonza's negligence or intentional misconduct; or (b) results from a critical failure at the Facility or in the performance of the Manufacturing Process or the equipment used to manufacture Product that, in each case, is primarily attributable to Lonza's breach of its obligations hereunder or Lonza's negligence or intentional misconduct.
"Manufacturing Process"	means the production process for the manufacture of Product, as such process may be improved or modified from time to time by agreement of the Parties in writing.
"Master Batch Record"	means the document, proposed by Lonza and approved by Customer, which defines the manufacturing methods, test methods and other procedures, directions and controls associated with the manufacture and testing of Product.
"Maximum Capacity"	means with respect to the Dedicated Production Line, the maximum number of Batches that can be produced in any 12-month period (assuming overlapping consecutive runs after process validation), which, unless otherwise mutually agreed by the Parties in writing, shall be: (a) […***…] Batches during the period from completion of the last Process Validation Batch until the earlier of (i) the […***…] anniversary of First Approval and (ii) expiration of the […***…] Request Period (as defined in Clause 9.2); (b) […***…] Batches during the period from expiration of the […***…] Request Period until the earlier of (i) the […***…] anniversary of First Approval and (ii) expiration of the […***…] Request Period (as defined in Clause 9.2) (c) […***…] Batches during the period from expiration of the […***…] Request Period until the […***…] anniversary of First Approval;

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	in each case, subject to: (x) pro rata adjustment for any 12-month period during which the Batch production level increases or decreases as described in Clause 3.3; and (y) reduction in the event of Customer's exercise of its right to decrease the Batch production level in accordance with Clause 3.3.6.
"Measurement Period"	means a period of 12 calendar months beginning on the first day of the calendar month following: (i) with respect to the initial Measurement Period, Release of the final Validation Batch; and (ii) with respect to subsequent Measurement Periods, (A) the end of the Target Fulfillment Period with respect to the previous Measurement Period if the Minimum Production Target was not met for such previous Measurement Period, otherwise, (B) the end of the previous Measurement Period.
"Measurement Period Notice"	has the meaning given in Clause 9.9.1.
"Minimum Production Rate"	has the meaning given in Appendix D.
"Minimum Production Target"	has the meaning given in Appendix D.
"Monthly […***…] Fee"	has the meaning given in Clause 9.2.
"Monthly […***…] Fee"	has the meaning given in Clause 9.2.
"New Customer Intellectual Property"	has the meaning given in Clause 10.2.
"New General Application Intellectual Property"	has the meaning given in Clause 10.3.
"Operational Failure"	means the suspension by Lonza of production of Product for more than […***…] ([…***…]) days due to the occurrence of a failure at the Facility or in the equipment used to the manufacture Product (and which failure is not attributable to […***…]) at any time following the Engineering Run Phase. Operational Failure would include, without limitation, such a suspension by Lonza due to […***…].
"PAI"	means Pre-Approval Inspection as defined by the FDA or EMA.
"Party"	means each of Lonza and Customer and, together, the "Parties".

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"Persistent Supply Failure"	means: (i) an Operational Failure exists and has existed for a period of […***…] ([…***…]) consecutive calendar months; or (ii) a Target Fulfillment Period is ongoing and has been ongoing for at least […***…] ([…***…]) calendar months.
"Process Validation Batch"	means a Batch that is produced with the intent to show reproducibility of the Manufacturing Process and is required to complete process validation studies.
"Product"	means Customer's proprietary AAV1/SERCA2a drug substance, an adenovirus-associated virus based vector containing the expression cassette for SERCA2a product.
"Product Warranty"	has the meaning provided in Clause 11.1.2.
"Production Clean Room"	means a clean room intended for use in cell culture, production of viral product, viral purification and/or non-viral purification.
"Project Plan"	means the plan(s) describing the Services to be performed by Lonza under this Agreement, including any update and amendment of such plans to which the Parties may agree from time to time. The initial Project Plan(s) are attached hereto as Appendix A.
"Purchase Order"	means a purchase order submitted by Customer in accordance with Clause 7.2 (to the extent consistent with Forecasts delivered by Customer pursuant to Clause 7.1) for one or more cGMP Batches.
"Quality Agreement"	means the quality agreement to be mutually agreed by the Parties before commencement of cGMP manufacturing and to be attached hereto as Appendix F (which will be incorporated herein by reference), and that will set out the responsibilities of the Parties in relation to quality as required for compliance with cGMP.
"Raw Materials"	means all ingredients, reagents, solvents, any other components of the Product as well as consumables and wearables, required to perform the Manufacturing Process or Services set forth in the bill of materials detailing the same (including Downstream Materials).
"Raw Materials Fee"	means the procurement and handling fee of […***…] percent ([…***…]%) of the acquisition cost of Raw Materials by Lonza that is charged to the Customer in addition to the cost of such Raw Materials, except for Downstream Materials

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	which will be subject to the Downstream Materials Handling Fee.
"Regulatory Authority"	means the FDA, EMA and any other similar regulatory authorities as may be agreed upon in writing by the Parties.
"Release"	means, with respect to any cGMP Batch, the release of such cGMP Batch by Lonza Quality Assurance in accordance with the Quality Agreement.
"Reservation Period"	means, collectively, the Initial Reservation Period (as defined in Clause 2.1) and the extension period(s) for which Customer pays the applicable extension fee pursuant to Clauses 2.2, 2.3 and/or 2.4.
"Services"	means all or any part of the services to be performed by Lonza under this Agreement (including, without limitation, process and analytical method transfer, facility construction, process validation, clinical and commercial manufacturing, as well as quality control and quality assurance activities), particulars of which are set out in a Project Plan and Purchase Orders, Master Batch Records or Quality Agreement, as applicable.
"Shared Resources"	means any system, material, input, output, service or resource within the Building or Facility or associated with the Services, which is not exclusively dedicated to the Product or the Manufacturing Process hereunder. By way of illustration, Shared Resources include but are not limited to: quality control laboratories, manufacturing sciences laboratories, office/administration space, electricity, WFI water, disposal, buffer preparation, security, site IT, process controls, DeltaV systems, shipping/receiving, storage, and raw material handling.
"Specifications"	means the specifications of the Product to be mutually agreed by the Parties before commencement of the production run for the first Engineering Batch and to be attached hereto as Appendix B (which will be incorporated herein by reference), which may be amended from time to time in accordance with this Agreement.
"Target Fulfillment Period"	means with respect to any Measurement Period for which the applicable Minimum Production Target has not been met, a period of

	consecutive calendar months following such Measurement Period, during which Lonza will continue to manufacture Batches until it has successfully made and Delivered additional Batches ([…***…]) that, together with Batches successfully made and Delivered during such Measurement Period ([…***…]), achieve a Minimum Production Rate that meets the Minimum Production Target for such Measurement Period. Each Target Fulfillment Period will end on the last day of the calendar month in which manufacturing and Delivery of such additional Batches is completed.
"Term"	has the meaning given in Clause 14.1.
"Third Party"	means any party other than Customer, Lonza and their respective Affiliates.

In this Agreement references to the Parties are to the Parties to this Agreement, headings are used for convenience only and do not affect its interpretation, references to a statutory provision include references to the statutory provision as modified or re-enacted or both from time to time and to any subordinate legislation made under the statutory provision, references to the singular include the plural and vice versa, and references to the word "including" are to be construed without limitation.

2 Reservation Period and Construction Trigger

2.1 Initial Reservation Period. On or before the third (3rd) business day after the Effective Date, Customer shall pay Lonza a non-refundable reservation fee for the period beginning on the Effective Date and ending on […***…] (the "Initial Reservation Period") of $1,000,000 (the "[…***…] Reservation Fee"). In the event Customer fails to timely pay the 2014 Reservation Fee, this Agreement will automatically terminate as of 11:59 pm on the fifth (5th) business day following the Effective Date.

2.2 First Extension of Reservation Period. If Customer has not exercised the Construction Trigger by […***…], Customer shall have the right, in its sole discretion, to extend the Reservation Period by an additional […***…] ([…***…]) months to […***…] upon notice to Lonza by […***…] along with payment of a non-refundable reservation extension fee of an additional $[…***…] (the "First Extension Fee").

2.3 Second Extension of Reservation Period. If Customer has not exercised the Construction Trigger by […***…], Customer shall have the right, in its sole discretion, to extend the Reservation Period by an additional […***…] ([…***…]) months to […***…] upon notice to Lonza by […***…] along with payment of a non-refundable reservation extension fee of an additional $[…***…] (the "Second Extension Fee").

2.4 Third Extension of Reservation Period. If Customer has not exercised the Construction Trigger by […***…], Customer shall have the right, in its sole discretion, to extend the Reservation Period by an additional […***…] ([…***…]) months to […***…] upon notice to Lonza given by […***…] along with payment of a non-refundable reservation extension fee of an additional $[…***…] (the "Third Extension Fee").

2.5 Credit for Extension Fees. If Customer exercises the Construction Trigger in accordance with Clause 2.6, Customer shall be entitled to a credit towards future Milestone Payments and pre-construction activities set forth in Appendix A-2 in an amount equal to the sum of the following: (a) if Customer paid the First Extension Fee prior to exercise of the Construction Trigger, […***…]% of the First Extension Fee ($[…***…]); (b) if Customer paid the Second Extension Fee prior to exercise of the Construction Trigger, […***…]% of the Second Extension Fee ($[…***…]); and (c) if Customer paid the Third Extension Fee prior to exercise of the Construction Trigger, […***…]% of the Third Extension Fee ($[…***…]).

2.6 Construction Trigger. Customer shall have the right, exercisable by written notice to Lonza delivered at any time during the Reservation Period, but not prior to completion of the Detailed Design, to require Lonza to commence construction of the Facility as described in Clause 3.2 (the "Construction Trigger"). Within five (5) business days following Customer's exercise of the Construction Trigger:

2.6.1 Lonza shall purchase directly from Customer a number of shares of Customer's common stock equal to the lesser of (i) that number of shares having an aggregate purchase price equal to, as closely as possible without exceeding based on the Per Share Purchase Price, $10,000,000 at the Per Share Purchase Price and (ii) one share less than the lowest number of shares that would exceed the lower of the Nasdaq Share Caps. The foregoing sale and purchase of Customer's shares of common stock shall be made pursuant to a Common Stock Purchase Agreement in substantially the form attached hereto as Appendix G, which shall be entered into by the Parties on or before the third (3rd) business day following the date on which the Construction Trigger is exercised.

For purposes of this Clause 2.6.1:

(a) "Nasdaq 20% Issuance Cap" means, if the Per Share Purchase Price is less than the greater of book or market value of Customer's common stock on the date the Construction Trigger is exercised, the number of shares that would result in the issuance of more than 19.99% of Customer's outstanding common stock as of immediately before the issuance (in each case, as calculated in accordance with the rules and guidance of The NASDAQ Global Market).

(b) "Nasdaq Change of Control Cap" means the number of shares that would result in Lonza beneficially owning, immediately following the issuance of Customer's common stock contemplated by the initial paragraph of this Clause 2.6.1, in excess of 19.99% of the outstanding common stock or voting power of Customer (as calculated in accordance with the rules and guidance of The NASDAQ Global Market).

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(c) "Nasdaq Share Caps" means, collectively, the Nasdaq 20% Issuance Cap and the Nasdaq Change of Control Cap.

(d) "Per Share Purchase Price" means a price per share that is equal to the average of the Volume Weighted Average Prices per share of Customer's common stock for the […***…] trading days immediately preceding the date on which Customer exercises the Construction Trigger.

(e) "Volume Weighted Average Price" means the volume weighted average sale price of Customer's common stock on The Nasdaq Global Market (without regard to after-hours trading or any other trading outside of the regular trading hours) as reported by Bloomberg Financial Markets or an equivalent, reliable reporting service agreed upon in writing by the Parties.

2.6.2 Lonza, Customer and the Escrow Agent shall execute and deliver the Escrow Agreement; and

2.6.3 subject to execution of the Escrow Agreement, Customer […***…] to the […***…] ($[…***…]); provided, that Customer may in any event delay deposit of such additional […***…] dollars ($[…***…]) for up to […***…] ([…***…]) days after the date on which the Construction Trigger is exercised.

2.6.4 If Customer executes the Agreement and simultaneously exercises the Construction Trigger, the reservation and extension fees described in this Clause 2 shall not apply.

3 Performance of Services

3.1 Performance of Services Generally. Following exercise of the Construction Trigger, and subject to completion of the transactions described in Clauses 2.6.1 through 2.6.3, Lonza shall itself and through its Affiliates:

3.1.1 use commercially reasonable efforts to perform the Services under each Project Plan in accordance with the terms and conditions of this Agreement on the estimated timelines as set forth in the applicable Project Plan; and

3.1.2 use commercially reasonable efforts to manufacture cGMP Batches under Purchase Orders in accordance with the terms and conditions of this Agreement on the production schedules delivered by Lonza to Customer pursuant to this Agreement.

Lonza shall be entitled to have one or more of its Affiliates perform any of Lonza's obligations contained in this Agreement. Lonza shall remain fully responsible for its Affiliates' performance of all obligations delegated to Affiliates. Lonza shall retain appropriately qualified and trained personnel with the requisite knowledge and experience to perform the Services in accordance with this Agreement. Lonza may subcontract or delegate any of its rights or obligations under this Agreement to perform the Services to Third Party subcontractors with Customer's prior written consent, which shall not be unreasonably withheld or delayed; provided, that Lonza shall be responsible for the acts or omissions of such Third Party subcontractors.

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Notwithstanding the foregoing, Lonza shall not be responsible for erroneous results of analytical lab services performed by External Laboratories approved by Customer.

3.2 Facility Construction and Technology Transfer.

3.2.1 Within […***…] ([…***…]) days after the Construction Trigger (subject to completion of the transactions described in Clause 2.6), Lonza will initiate construction of the Facility within the Building.

3.2.2 Lonza will use reasonable efforts to initiate pre-construction activities as described in the applicable Project Plan prior to exercise of the Construction Trigger to enable construction to be initiated on the schedule described in Clause 3.2.1.

3.2.3 Thereafter, Lonza will undertake the construction of the Facility (including a Dedicated Production Line and Shared Resources), in accordance with the Detailed Design and Lonza procedures and policies, and use commercially reasonable efforts to complete such construction on the estimated timeline for construction of the Facility attached hereto as Appendix C.

3.2.4 The estimated […***…] is or will be described in the […***…] (such total estimated […***…] is referred to herein as the "CapEx Amount" or "CapEx"). Lonza shall provide […***…] for […***…], to the extent described in the […***…], and Lonza (or its Affiliate, in Lonza's sole discretion) shall […***…], including the […***…] and […***…] in connection with the performance of the Services and operation of the Dedicated Production Line. Any mutually-agreed changes to the scope of the […***…] shall be […***…].

3.2.5 Lonza shall transfer the Manufacturing Process to the Facility, including implementing the technology transfer plan set forth in the applicable Project Plan. Customer shall provide reasonable assistance and support to Lonza in implementing such technology transfer as reasonably requested by Lonza.

3.2.6 Any Capital Equipment required for the performance of the Services but not included in the Detailed Design shall be acquired on terms to be agreed by the Parties prior to commencement of the relevant Services.

3.3 Production Level

3.3.1 Start-Up and Initial Staffing. Lonza shall perform the Facility commissioning and start up to include the recruitment and training of qualified personnel required to operate the Dedicated Production Line and perform the Services at the Facility. Lonza shall initially recruit and train sufficient staff at the Facility to perform the initial shake down runs and the process validation/conformance lots through Release of the final Validation Batch.

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3.3.2 Staffing Generally. Lonza shall staff the Facility consistent with the staffing schedules provided to Customer following commencement of the first Measurement Period.

3.3.3 […***…]-Batch Production Level. Upon Release of the final Validation Batch, Lonza shall have recruited and trained sufficient staff at the Facility to supply up to […***…] Batches per year (assuming overlapping consecutive runs after process validation).

3.3.4 […***…]-Batch Production Level. Customer may request to increase the production level of the Facility to be able to supply up to […***…] Batches per year (assuming overlapping consecutive runs after process validation) ("[…***…] Batch Request"). Lonza agrees to be up to the […***…] Batch/year production level within […***…] months following the date of Customer's […***…] Batch Request.

3.3.5 […***…]-Batch Production Level. Customer may request to increase the production level of the Facility to be able to supply up to […***…] Batches per year (assuming overlapping consecutive runs after process validation) ("[…***…] Batch Request"). Lonza agrees to be up to the […***…] Batch/year production level within […***…] months following the date of Customer's […***…] Batch Request.

3.3.6 Decreasing Production Level. Customer may request to decrease the production level (and associated monthly suite fee) from […***…] Batches/year to […***…] Batches/year or from […***…] Batches/year to […***…] Batch/year by giving Lonza […***…] months' notice; provided that […***…].

3.3.7 Other Production Level Changes. In the event Customer desires to change the production level otherwise than according to the above provisions, both parties agree to negotiate timings and monthly suite fees in good faith, provided that the timing of achieving production rates (ramp up or down) and monthly suite fees will be based on the foregoing provisions and Clause.

3.3.8 Dedicated Production Line; Other Clean Rooms. During the Term, Lonza will not use the Dedicated Production Line except to provide Services hereunder. Lonza may, at its own expense, build-out and operate additional Production Clean Rooms within the Building for use in providing services to customers other than Customer, provided that Lonza shall at all times ensure that any such build-out does not impair Lonza's ability to comply with its obligations hereunder. If at any time during the Term, Lonza desires to expand the Building or production areas within the Building beyond what is otherwise agreed with Customer under this Agreement, Lonza shall give prompt notice thereof to Customer, and the parties shall discuss in good faith regarding Customer becoming an exclusive or non-exclusive customer with respect to production areas to be included in such expansion.

3.4 Engineering Batches. Following completion of Facility construction, commissioning and startup, Lonza shall manufacture Engineering Batches until it has completed […***…] ([…***…]) successful Engineering Batches, in compliance with cGMP and

Specifications, unless the parties mutually agree that a lesser number of successful Engineering Batches has sufficiently demonstrated that the Manufacturing Process has been successfully transferred to the Facility and can be operated therein in compliance with cGMP. Customer shall have the right to make whatever further use of the non-cGMP Engineering Batches as it shall determine, provided that such use is not for human use and does not violate any Applicable Laws. Lonza makes no warranty that Engineering Batches will meet cGMP or the Specifications. If Lonza determines that an Engineering Batch does meet cGMP and the Specifications, it will release such Engineering Batch as a cGMP Batch. Regardless of whether any Engineering Batch meets cGMP or the Specifications, Customer shall pay to Lonza the Raw Materials Fee associated with such Engineering Batches.

3.5 cGMP Batches. Lonza will, in accordance with the terms of this Agreement and Quality Agreement, manufacture at the Facility and Release to Customer, cGMP Batches that comply with the Manufacturing Process, cGMP and the Specifications, together with a Certificate of Analysis; *provided, however,* that cGMP manufacture shall not commence until Lonza has completed the Engineering Batches described in Clause 3.4. Prior to commencement of cGMP manufacturing, the Steering Committee shall review the process assumptions. In the event that there is a material difference in the process assumptions as compared with the process results demonstrated during the manufacture of Engineering Batches, the Parties shall meet to discuss in good faith a revision to the fee structure hereunder to reflect such difference. Any such revision would be effective only upon execution by the Parties of a written amendment to this Agreement on mutually acceptable terms.

3.6 Process Validation Batches. Lonza shall manufacture and deliver Process Validation Batches as mutually agreed by Parties sufficient to document the operability and reproducibility of the Manufacturing Process and permit the Parties to complete and file the necessary regulatory documents.

3.6.1 Prior to commencement of Process Validation Batches, Lonza and Customer shall agree a process validation plan identifying the validation requirements of the Manufacturing Process. Certain process validation activities, other than manufacture of Process Validation Batches, may be excluded from the monthly suite fee structure hereunder and shall be paid for by the Customer at the price set out in the applicable Project Plan.

3.6.2 Any regulatory support activities (including pre-Approval inspection) required and agreed to by Customer to support the Approval of the Product from the Facility shall be performed and supported by Lonza as reasonably requested by Customer. All such regulatory support activities are excluded from the monthly suite fee structure, shall be approved by the Customer in advance, and shall be paid for by the Customer at the price set out in the applicable Project Plan.

3.7 Supply of Customer Information and Customer Materials. Customer shall supply to Lonza all Customer Information and Customer Materials and other information or materials that may be reasonably required by Lonza to perform the Services other than such information and materials that are in the possession of Lonza Houston, Inc. Lonza shall not be responsible for any delays arising out of Customer's failure to provide such Customer Information, Customer Materials, or other information or materials reasonably required to perform the Services to Lonza, and Customer shall be responsible for all additional costs and expenses arising out of such delay,

excluding any idle Facility capacity costs (which the Parties acknowledge is already factored into the monthly suite fees under this Agreement).

3.8 Raw Materials. Lonza shall procure all required Raw Materials as well as consumables other than those Raw Materials that are Customer Materials. Safety stock quantities of Raw Materials as well as Downstream Materials will be mutually agreed between Lonza and Customer and will be purchased at Customer's expense and invoiced when ordered (acquisition cost plus applicable handling fees). Customer shall be responsible for payment for all Raw Materials and Downstream Materials ordered or irrevocably committed to be procured by Lonza hereunder. Upon cancellation of any Batch or termination of the Agreement, all unused Raw Materials shall be paid for by Customer within thirty (30) days of invoice and at Customer's option will either be (a) held by Lonza for future use for the production of Product, (b) delivered to Customer, (c) returned to the supplier, to the extent permitted by the supplier, or (d) disposed of by Lonza. Lonza will credit Customer for any credits received by Lonza in connection with the return of Raw Materials to the supplier.

4 Project Management / Steering Committee

4.1 Project Plans. With respect to a new project to be governed by this Agreement, a new Project Plan shall be added by agreement in a writing signed by the Parties and appended to Appendix A. Each Project Plan shall include a description of the Services to be provided, the Product to be manufactured, Specifications, a schedule for completion of the Project Plan, pricing details (if not already covered by the fee structure under this Agreement), and such other information as is necessary for relevant Services. The Parties shall discuss and reach mutual agreement on the budget for such Project Plan, including agreement with respect to the amount of such costs that are already covered by the fee structure under this Agreement. In the event of a conflict between the terms of a Project Plan and this Agreement, the terms of this Agreement will govern. Each Party shall at all times exercise good faith and be commercially reasonable in negotiating Project Plans.

4.2 Project Management; Dispute Resolution. With respect to each Project Plan, each party will appoint a project manager who will be the party responsible for overseeing the Project Plan. The project managers designated by each party will first attempt to resolve any disputes within […***…]. If the project managers are unable to do so, then the dispute will be elevated to the Steering Committee.

4.3 Steering Committee. Each Party shall name a mutually agreed upon equal number of senior management representatives for the Steering Committee, which shall meet at least quarterly, with one of such meetings each calendar year to be held at a Lonza site located in the U.S. and one of such meetings each calendar year to be held at Customer's site, or as otherwise mutually agreed by the Parties. In the event that Customer grants a Third Party any license or rights to Product in the U.S. or the European Union, then subject to execution of an appropriate confidentiality agreement between such Third Party and Lonza, Customer may, but need not, permit such Third Party to be represented on the Steering Committee or to participate in Steering Committee meetings as a non-voting participant, at Customer's sole discretion. In the event that a Steering Committee is unable to resolve any dispute within […***…], then such dispute shall be escalated to a senior executive of each of Customer and Lonza for attempted resolution. If such senior executives are unable to resolve such dispute within […***…] of such escalation, then either party may pursue any and all remedies available at law or in equity.

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The primary function of the Steering Committee is to ensure the ongoing communication between the Parties (including, without limitation, ongoing disclosure by Lonza to Customer regarding the status and progress of development of the Detailed Design, any anticipated changes or updates to the […***…], and the estimated timing of Facility Mechanical Completion and Facility OQ) and discuss and resolve any issues arising under this Agreement. In addition to the primary function described above, the Steering Committee shall also take on the following responsibilities:

4.3.1 discuss and seek resolution of issues around management of the Services, including without limitation the ability and availability of Facility staff to perform non-manufacturing Services included in Project Plans hereunder;

4.3.2 monitor deadlines and milestones for the Services; and

4.3.3 discuss and recommend any changes to the Services (although such changes will not take effect until they have been incorporated into a written amendment to the Project Plan which has been signed by the Parties).

4.4 Person in Plant. Beginning […***…] Batch in the Facility, Customer shall be permitted to have, […***…] ([…***…]) employees in the clean rooms included in the Dedicated Production Line as reasonably requested by Customer, for the purpose of observing, reporting on, and consulting as to the performance of the Services. Such employees shall be subject to and agree to abide by Lonza's customary practices and operating procedures regarding persons in plant, and such employees agree to comply with all instructions of Lonza's employees at the Facility.

5 Quality

5.1 Responsibility for quality assurance and quality control of Product shall be allocated between Customer and Lonza as set forth in the Quality Agreement and in Lonza standard operating procedures. If there is a conflict between the terms and conditions of this Agreement and the Quality Agreement, the terms and conditions of the Quality Agreement shall control with respect to matters related to coordinating Product quality control and quality assurance activities, and this Agreement shall prevail in all other cases. If the Quality Agreement is not in place at the Effective Date, Lonza and Customer commit to enter into the Quality Agreement in a timely manner, but in no event later than the commencement of cGMP manufacturing.

5.2 For clarity and without limiting any corresponding obligations in the Quality Agreement, Lonza shall provide prompt written notice to Customer upon receipt from any Governmental Authority of a warning letter or consent decree with respect to the Facility.

5.3 Provisions regarding inspections by Regulatory Authorities and audits shall be set out in the Quality Agreement.

6 Insurance

6.1 Each Party shall, during the Term and for five (5) years after delivery of the last Product manufactured or Services provided under this Agreement, obtain and

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maintain at its own cost and expense from a qualified insurance company, insurance coverage in the following amounts:

6.1.1 with respect to Lonza, comprehensive general liability insurance including, but not limited to product liability coverage in the amount of at least […***…] ([…***…]) […***…] dollars per claim and in the aggregate; and

6.1.2 with respect to Customer, at least (a) […***…] ([…***…])[…***…] dollars per occurrence and in the aggregate of product liability insurance and (b) at least […***…] ([…***…]) million dollars per occurrence and […***…] ([…***…]) million dollars in the aggregate of general liability (non-product liability) insurance.

6.2 In addition, if Customer exercises the Construction Trigger, then (a) Lonza shall promptly, but in any event within thirty (30) days following the date of Construction Trigger, obtain and maintain for the duration of the construction at its own cost and expense from a qualified insurance company, a builder's risk policy on the Facility in an amount equal to at least the […***…]; and (b) upon Facility OQ and for the remainder of the Term, Lonza shall obtain and maintain at its own cost and expense from a qualified insurance company, property coverage on the Facility, in an amount equal to at least […***…]. In the event that, as a result of Force Majeure, the Facility is damaged but repairable, then, unless otherwise mutually agreed by the Parties in writing, all insurance proceeds from the applicable policy shall be used for repair (and further construction, if applicable) of the Facility. In the event that, as a result of Force Majeure, the Facility is destroyed, or damaged to such an extent that construction of a new facility would be required, then:

6.2.1 if the Parties mutually agree in writing to construct a new facility, all insurance proceeds from the applicable policy shall be applied to such construction; and

6.2.2 if the Parties do not mutually agree in writing to construct a new facility, then the insurance proceeds from the applicable policy shall be allocated as follows:

(a) if Lonza's total […***…] as of the date of the Force Majeure event exceed the aggregate milestones paid to Lonza from the Escrow Account to such date, the proceeds will be used first, to reimburse Lonza for the amount of such excess, and second, to reimburse Customer for the aggregate milestones […***…] to such date, and any remaining proceeds will be retained by Lonza.

(b) if the aggregate milestones paid to Lonza from the Escrow Account to such date exceed Lonza's […***…] as of the date of the Force Majeure event, the proceeds will be used first to reimburse Customer for the […***…] to such date, and any remaining proceeds will be retained by Lonza.

6.3 The foregoing provisions of this Clause 6.2 are in addition to any termination right that Customer may have under Clause 15.1 as a result of such Force Majeure event. Each Party shall ensure that the other Party is named as an additional insured or loss

payee, as applicable, on such insurance and provide the respective other Party with a certificate of such insurance upon reasonable request.

6.4 If Customer exercises the Construction Trigger, Lonza shall, within thirty (30) days following the date of Construction Trigger, provide Customer with a worker's compensation certificate containing a waiver of subrogation in favor of Customer and proof of auto liability coverage, and shall update such coverage as additional FTEs are assigned to the projects covered by Project Plans.

7 Forecasting, Ordering and Cancellation

7.1 Forecasting. No later than the first (1st) day of each calendar quarter, Customer shall supply Lonza with a written forecast showing Customer's good faith estimated quarterly requirements for Batches for the following […***…] ([…***…]) month period (the "Forecast"), which in no event shall exceed the Maximum Capacity for any calendar year. No later than […***…] ([…***…]) business days following Lonza's receipt of a Forecast, Lonza shall provide Customer with an estimated production schedule showing the estimated Commencement Date and Release date of each Batch. The forecast given in this Clause 7.1 shall not be binding on Customer or Lonza, except that Customer shall submit orders for Batches scheduled to commence within the first […***…] ([…***…]) months of each Forecast in order ensure that Raw Materials can be ordered in a timely manner.

7.2 Purchase Orders/Schedule. Each Purchase Order must be submitted at least […***…] ([…***…]) months prior to the earliest Commencement Date of the Batches covered by such Purchase Order (as estimated by Lonza pursuant to Clause 7.1).

7.3 Shortfall Notice. If at any time during any calendar year following commencement of cGMP manufacture of Product at the Facility, Lonza believes that it will be unable to Deliver in such calendar year the number of Batches ordered by Customer for Delivery during such calendar year, Lonza shall promptly provide written notice thereof to Customer, which notice shall include (i) the number of Batches that Lonza believes it will be unable to Deliver, (ii) the reasons for Lonza's inability to deliver such number of Batches and (iii) Lonza's anticipated timeline for being able to deliver such number of Batches (such notice, a "Shortfall Notice"). Following delivery of a Shortfall Notice, Lonza shall be obligated to provide written notice(s) to Customer promptly in the event there are subsequent changes in the details covered by a particular Shortfall Notice (e.g., if Lonza subsequently learns that it will be able to Deliver more or less Batches than previously described in the Shortfall Notice or any prior update notice, or if Lonza's anticipated timelines for curing such shortfall change). For clarity, the provisions of this Clause 7.3 and Clause 7.4 and other provisions relating to mechanisms addressing Lonza's inability to Deliver Batches set forth in Purchase Orders under this Agreement shall not limit or otherwise affect Lonza's obligations to continue efforts to manufacture and Deliver Products in accordance with Clause 3.1 of this Agreement.

7.4 Minimum Quantity.

7.4.1 Subject to Clause 7.4.2, during each calendar year after commencement of cGMP manufacture of Product at the Facility during which Customer is paying less than the Monthly […***…] Fee (other than due to reduction of monthly suite fees as provided under Clause 9.9 of this Agreement), Customer will place orders hereunder for Batches comprising not less than […***…]% of the world-wide Product requirements of Customer and its strategic partners

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and licensees. Notwithstanding the foregoing, in the event Customer desires Product sooner than Lonza is able to supply it under this Agreement Customer shall have the right to purchase and obtain Products from Third Party suppliers until cGMP manufacture of Product commences at the Facility up to the quantities reasonably necessary for Customer to obtain during such period, and Customer shall notify Lonza in writing of the quantities it orders from such Third Party suppliers.

7.4.2 If at any time during any calendar year following commencement of cGMP manufacture of Product at the Facility, Lonza provides a Shortfall Notice to Customer or Lonza does not Deliver in such calendar year the number of Batches ordered by Customer for Delivery during such calendar year, whichever occurs first, then immediately upon the occurrence of such event, notwithstanding Clause 7.4.1, Customer shall have the right to order and have supplied from any Third Party (a) that number of Batches that Lonza is unable to Deliver (or so indicates in a Shortfall Notice or update thereto) (the "Shortfall"); and (b) that number of Batches equal to the product of (i) Customer's total Batch requirements for the first […***…] ([…***…]) months of the calendar year following the calendar year in which the Shortfall occurred, multiplied by (ii) a proportion, the numerator of which is the total number of Batches included in the Shortfall during the preceding calendar year and the denominator of which is the total number of Batches set forth in Purchase Orders which should have been Delivered in the preceding calendar year (including the Batches included in the Shortfall). For clarity, in the event Customer elects to purchase Batches from any Third Party pursuant to this Clause 7.4.2, the Products included in such Batches actually purchased shall not be included in Customer's and its strategic partners' and licensees' total Product requirements for purposes of the calculation described in Clause 7.4.1. Customer's right to purchase Batches from a Third Party as described in this Clause 7.4.2 shall apply regardless of whether Lonza subsequently Delivers the Shortfall Batches or is able to meet Customer's Product requirements for the first […***…] ([…***…]) months after the calendar year in which the Shortfall occurred.

8 Delivery and Acceptance

8.1 Delivery. All Product shall be delivered EXW (as defined by Incoterms® 2010) the Facility. Following completion of the batch review procedures as set forth in the Quality Agreement (during which Customer and Lonza shall review and resolve comments pertaining to the executed batch records and related lot documentation), Lonza shall deliver to Customer a final Certificate of Analysis for each cGMP Batch and such other documentation as is reasonably required to meet all applicable regulatory requirements of the Governmental Authorities (collectively, the "Batch Documentation") within […***…] ([…***…]) business days after Release of such cGMP Batch. Lonza shall provide Customer with prompt written notice of Release of each cGMP Batch. With respect to any Customer Materials, title and risk of loss shall remain with the Customer and shall not transfer to Lonza. With respect to Product, title and risk of loss shall transfer to Customer upon Delivery in accordance with this provision, provided that if Lonza has not delivered the Batch Documentation for such Product to Customer prior to Delivery, then title and risk of loss shall transfer to Customer upon delivery of such Batch Documentation.

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8.2 Storage. Customer shall arrange for pickup of such Batch from the Facility, at Customer's expense, within […***…] days after Release or pay applicable storage costs. Lonza shall provide storage on a bill and hold basis for such Batch(es) at no charge for up to […***…] ([…***…]) days; provided that any additional storage beyond […***…] ([…***…]) days will be subject to availability and, if available, will be charged to Customer and will be subject to a separate agreement. In addition to Clause 8.2, Customer shall be responsible for all value added tax (VAT) and any other applicable taxes, levies, import, duties and fees of whatever nature imposed as a result of any storage. Notwithstanding anything to the contrary contained in this Agreement, in no event shall Lonza be required to store any Batch for more than […***…] ([…***…]) calendar days after Release. Within […***…] ([…***…]) days following a written request from Lonza, Customer shall provide Lonza with a letter in form satisfactory to Lonza confirming the bill and hold status of each stored Batch.

8.3 Acceptance/Rejection of Product.

8.3.1 Within […***…] ([…***…]) days after Customer's receipt of all Batch Documentation for a Batch, or such other period as may be specified in the Quality Agreement, Customer shall determine by review of such Batch Documentation whether or not such Batch conforms to the Product Warranty. Promptly following receipt of each Batch, Customer shall inspect such Batch. Customer shall have the right to reject any Batch that fails to conform to the Product Warranty. Customer shall notify Lonza in writing of any rejection of a Batch based on any claim of non-conformity to the Product Warranty within […***…] ([…***…]) days of Customer's receipt of the Batch, after which time such Batch shall be deemed accepted. Customer shall have the right to revoke its acceptance and reject a Batch if within […***…] ([…***…]) months following Release of such Batch, Customer discovers a latent defect in such Batch which Customer could not have reasonably discovered upon inspection of such Batch and the related Batch Documentation, and Customer provides written notice thereof to Lonza within […***…] ([…***…]) business days of discovery thereof.

8.3.2 In the event that Lonza believes that a Batch has been incorrectly rejected, Lonza may require that Customer provide to it Batch samples for testing. Lonza may retain and test the samples of such Batch. In the event of (a) a discrepancy between Customer's and Lonza's test results such that Lonza's test results fall within relevant Specifications, or (b) there exists a dispute between the Parties over the extent to which such failure is attributable to a given Party (including, without limitation, any such dispute over whether such failure is a Lonza Responsibility) that the Parties are unable to resolve within […***…] days of delivery by a Party to the other Party of written notice of the existence of such dispute, the Parties shall cause an independent laboratory promptly to review records, test data and perform comparative tests and/or analyses on samples of the Product that allegedly fails to conform to Specifications. Such independent laboratory and analytical procedures shall be mutually agreed upon by the Parties. The independent laboratory's results shall be in writing and shall be final and binding. Unless otherwise agreed to by the Parties in writing, the costs associated with such testing and review shall be borne by the Party against whom the independent laboratory rules.

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8.3.3 Lonza shall replace any cGMP Batch that does not conform to the Product Warranty (a "Failed cGMP Batch") with a cGMP Batch conforming to the Product Warranty. If a Failed cGMP Batch is a Lonza Responsibility, Lonza shall replace such Batch at no additional cost to Customer (except for the cost of Customer Materials). Such replacement shall be made as promptly as practicable. Customer acknowledges and agrees that, except in the case of Persistent Supply Failure (for which Customer shall have the rights and remedies expressly provided in this Agreement), Customer's sole remedy with respect to a Failed cGMP Batch is as set forth in this Clause 8.3.3. Lonza shall be responsible for the cost of Raw Materials (other than Customer Materials) consumed in any Failed cGMP Batch for which Lonza Responsibility has been established, but not otherwise.

8.3.4 Notwithstanding anything else in this Agreement, if the sole reason that any Failed cGMP Batch failed to conform to the Product Warranty was the failure of the Customer Materials provided by Customer for use in the manufacture of such Batch to conform to the applicable specifications for such materials, then, provided that Lonza performed quality testing and release on such Customer Materials in accordance with the Quality Agreement prior to their use, Lonza shall have no liability or responsibility for such Failed cGMP Batch under Clause 8.3.3.

9 Price and Payment

9.1 Milestones. Upon completion or occurrence of each of the Milestones set forth below, Lonza shall invoice Customer the amount set forth opposite such Milestone, less applicable credits posted to Customer's account pursuant Clause 2. Unless Customer notifies Lonza in writing that it disputes any such invoice within fifteen (15) business days following the date thereof, stating in reasonable detail the basis for disputing such invoice, Lonza shall be entitled to withdraw the amount of such invoice from the Escrow Account by presenting to the Escrow Agent a copy of the invoice evidencing completion of the applicable Milestone. If Customer timely disputes any such invoice, the matter will be resolved by the Steering Committee; provided that if the Steering Committee is unable to resolve such dispute within […***…] ([…***…]) days following the date of Customer's notice to Lonza, either Party may seek resolution of the matter pursuant to Clause 16.9. Lonza will not make a withdrawal from the Escrow Account as described above unless and until any dispute regarding the underlying invoice timely notified hereunder by Customer has been resolved in Lonza's favor. Customer will not give a "Counter Notice" under the Escrow Agreement unless it has timely notified Lonza that it disputes in good faith an invoice presented pursuant to this Clause, and such dispute has not been resolved in Lonza's favor. If Customer gives a Counter Notice under the Escrow Agreement and the underlying dispute is subsequently resolved in Lonza's favor, Customer will, upon request of Lonza, execute and deliver with Lonza a joint instruction to the Escrow Agent to disburse to the amount of the relevant Milestone Payment to Lonza.

Milestone	Milestone Payment amount
Customer's exercise of the Construction Trigger	[…***…]% of […***…]
Completion of the 1st engineering run, but no later than […***…] months after exercise of the	[…***…]% of […***…]

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Construction Trigger; provided that such […***…]- month period shall be extended by the duration of any delay in completion of the 1st engineering run caused by or within the reasonable control of Lonza	
Successful completion of a PAI, but no later than […***…] months after Facility OQ; provided that such […***…]-month period shall be extended by the duration of any delay in successful completion of the PAI caused by or within the reasonable control of Lonza	[…***…]% of […***…]
First Approval of commercial Product, but no later than […***…] months after Facility OQ; provided that such […***…]-month period shall be extended by the duration of any delay in First Approval of commercial Product caused by or within the reasonable control of Lonza	[…***…]% of […***…]
[…***…] months following First Approval of commercial Product, but no later than […***…] months after Facility OQ; provided that such […***…]-month period shall be extended by the duration of any delay in First Approval of commercial Product caused by or within the reasonable control of Lonza.	[…***…]% of […***…]
Payment of this Milestone Payment will be delayed until the date on which Lonza has supplied in accordance with this Agreement and the applicable Purchase Orders (1) the total number of Batches ordered by Customer for Delivery during the first […***…] ([…***…]) months following completion of final Process Validation Batch, or (2) […***…] Batches (including Process Validation Batches), whichever is less, but only if and to the extent the delay in production is caused by or within the reasonable control of Lonza.	

For purposes of the third milestone above and Clause 9.9.5 below, "Successful completion of a PAI" shall mean completion of a PAI and either (a) the FDA or EMA, as applicable, issues no comments as a result of the PAI, or (b) if the FDA or EMA, as applicable, issues comments as a result of the PAI, final remediation of all such comments by Lonza to the satisfaction of the FDA or EMA, as applicable.

In addition to the Milestones described above, if Lonza completes the 1st engineering run on or before […***…] ([…***…]) months after exercise of the Construction Trigger, Customer shall pay to Lonza a […***…]. Lonza shall notify Customer within thirty (30) days after such completion, and Customer will pay

Lonza such […***…] or notify Lonza in writing that it disputes such completion (stating in reasonable detail the basis for such dispute) within thirty (30) days after the date of such notice from Lonza. If Customer disputes such completion, the matter will be resolved by the Steering Committee; provided that if the Steering Committee is unable to resolve such dispute within thirty (30) days following the date of Customer's notice to Lonza, […***…]. For clarity, Customer shall not be required to […***…].

Notwithstanding any other provision in this Agreement, the Milestone payments made pursuant to this Clause 9.1 shall not be refundable for any reason and shall be paid only once.

9.2 Monthly Suite Fees. Customer shall pay monthly suite fees beginning upon Facility Mechanical Completion according to the schedule set forth below, subject to reduction in accordance with this Clause 9 and Appendix D. Lonza shall invoice Customer for the applicable monthly suite fee at the beginning of the calendar month for which it applies.

Period	**Monthly Suite Fee**
Facility Mechanical Completion to completion of Facility OQ	$[…***…] ("Monthly Start-up Fee")
Completion of Facility OQ through the […***…] anniversary of First Approval or until a different monthly suite fee becomes applicable hereunder	$[…***…] ("Monthly Base Fee")
[…***…] month period beginning […***…] months following request to increase Facility capacity to […***…] Batch/year production level ("[…***…] Request Period")	$[…***…]
End of […***…] Request Period through the [***] anniversary of First Approval or until a different monthly suite fee becomes applicable hereunder	$[…***…] ("Monthly […***…] Fee")* Based on […***…] FTEs, with adjustment subject to a lower limit of […***…] FTEs
[…***…] month period beginning […***…] months following request to increase Facility capacity to […***…] Batch/year production level ("[…***…] Request Period")	$[…***…]
End of […***…] Request Period through the […***…] anniversary of First Approval or until a different monthly suite fee becomes applicable hereunder	$[…***…] ("Monthly […***…] Fee")* Based on […***…] FTEs, with adjustment subject to a lower limit of […***…] FTEs

* Subject to potential reduction based on re-evaluation of applicable staffing plan to reduce FTEs as mutually agreed in good faith, but not below the lower limit specified above, in which case monthly suite fee would automatically be reduced by $[…***…] per FTE reduction. The foregoing suite fee reduction would not apply with respect to staff reductions resulting from Facility improvements made at Lonza's cost.

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Lonza may perform certain non-production Services outlined in each Project Plan, at […***…], Portsmouth, NH, and/or at other Lonza sites or facilities located in Portsmouth, NH, but there shall be no additional charges for such Services (outside of monthly suite fees described above) except to the extent such charges are described in the applicable Project Plan. Monthly suite fees do not include: (a) shipment, insurance, taxes, duties, and VAT that may be applicable, (b) Raw Materials Fees, costs of Downstream Materials and Downstream Materials Handling Fees, (c) costs from External Laboratories (if applicable) and any other Third Party contractors which will be passed through to Customer with a […***…]% management fee for sample preparation, results processing and review, (d) any unplanned capital expenditures not accounted for in Facility Construction, (e) all regulatory support services, (f) validation services (other than manufacturing of Process Validation Batches) that cannot reasonably be performed by the staff assigned to the Facility. All such validation work will be authorized in writing in advance by Customer and will be paid for by Customer at specified rates mutually agreeable with Lonza. Lonza will plan to reasonably use the Facility staff to meet objectives and deliverables as part of a cooperative Master Validation Plan with Lonza Houston Inc. All Process Validation and related activities performed by Lonza Houston Inc. employees will be contracted separately.

9.3 Unless otherwise indicated in writing by Lonza, all prices and charges are exclusive of value added tax (VAT) and of any other applicable taxes, levies, import, duties and fees of whatever nature imposed by or under the authority of any government or public authority and all such charges applicable to the Services shall be paid by Customer. When sending payment to Lonza, the Customer shall quote the relevant invoice number in its remittance advice.

9.4 Charges for Raw Materials (other than Downstream Materials) and the Raw Materials Fee for each Batch shall be invoiced upon the Release of each Batch. Charges for Downstream Materials shall be invoiced by Lonza upon placement of purchase orders for such Downstream Materials by Lonza at cost plus the Downstream Material Handling Fee.

9.5 Any additional Services contracted by Customer that are not included in the monthly suite fees (such as regulatory support activities) will be invoiced as provided in the applicable Project Plan.

9.6 All invoices are strictly net and payment must be made within thirty (30) days of date of invoice. Payment shall be made without deduction, deferment, set-off, lien or counterclaim.

9.7 If in default of payment of any undisputed invoice on the due date, interest shall accrue on any amount overdue at the rate of […***…] percent ([…***…]%) per month on a day to day basis until full payment; and Lonza shall, at its sole discretion, and without prejudice to any other of its accrued rights, be entitled to suspend the provision of the Services and or delivery of Product until all overdue amounts have been paid in full including interest for late payments.

9.8 Price adjustments.

9.8.1 Not more than […***…], and beginning no earlier than […***…], Lonza may adjust the monthly suite fees hereunder in accordance with the […***…] Index,

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[…***…] (or any successor index) increase for the previous calendar year.

9.8.2 In addition to the above, the […***…] may be […***…], to […***…]; provided, that Lonza (a) provides Customer with reasonable prior written notice of such […***…] (but in any event no less than 3 months' prior written notice); (b) together with the notice provided pursuant to clause (a), provides […***…] and […***…]; and (c) […***…] regarding such […***…] including providing any additional information regarding such […***…] as reasonably requested by Customer.

9.9 Other Reductions of Suite Fees.

9.9.1 Measurement Period Notice. If, at any time during a Measurement Period, Lonza determines that it will be unable to successfully manufacture during such Measurement Period the number of Batches required to meet the Minimum Production Target for such Measurement Period, Lonza shall promptly provide written notice thereof to Customer (such notice, a "Measurement Period Notice").

9.9.2 Reduction for Minimum Production Failure. Beginning in the first calendar month following delivery by Lonza of a Measurement Period Notice with respect to a Measurement Period during which it fails to meet the Minimum Production Target, but not later than the first calendar month of the Target Fulfillment Period with respect to such a Measurement Period, the applicable monthly suite fee shall be reduced (a) by […***…] percent ([…***…]%) through the […***…] ([…***…]) calendar month of such Target Fulfillment Period; and (b) beginning in the […***…] ([…***…]) calendar month of such Target Fulfillment Period, by […***…] percent ([…***…]%) until the end of such Target Fulfillment Period.

9.9.3 Operational Failure Reduction. If at any time an Operational Failure occurs, then during the Operational Failure, the applicable monthly suite fee shall be reduced by […***…] percent ([…***…]%); provided that, such reduction shall include (and not be in addition to) any reduction applied pursuant to Clause 9.9.2; and provided further that if Lonza Delivers, within the applicable calendar year, all Batches ordered by Customer for Delivery during each calendar year in which such Operational Failure exists, then Customer shall pay Lonza the total amount of the fee reductions applied pursuant to this provision as a result of such Operational Failure within thirty (30) days after Delivery of the last such Batch.

9.9.4 Reduction for Delay in Completion of 1st Engineering Run. If Lonza does not complete the 1st engineering run within […***…] months after exercise of the Construction Trigger, then the applicable monthly suite fee shall be reduced beginning in the next following calendar month until Lonza completes the 1st engineering run as follows: (a)

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the applicable monthly suite fee shall be reduced by […***…] percent ([…***…]%) through the […***…] ([…***…]) calendar month of such reduction; and (b) beginning in the […***…] ([…***…]) calendar month of such reduction, the applicable monthly suite fee shall be reduced by […***…] percent ([…***…]%), provided that such […***…]-month period shall be extended for, and such reductions shall not apply during, any period(s) of delay […***…]. The reduction set forth in this Clause 9.9.4 shall be in addition to any reduction applied pursuant to Clause 9.9.3.

9.9.5 Reduction for Delay in Completion of PAI. If Lonza does not successfully complete a PAI within […***…] months after Facility OQ, then the applicable monthly suite fee shall be reduced beginning in the next following calendar month until Lonza successfully completes a PAI as follows: (a) the applicable monthly suite fee shall be reduced by […***…] percent ([…***…]%) through the […***…] ([…***…]) calendar month of such reduction; and (b) beginning in the […***…] ([…***…]) calendar month of such reduction, the applicable monthly suite fee shall be reduced by […***…] percent ([…***…]%); provided that such […***…]-month period shall be extended for, and such reductions shall not apply during, any period(s) of delay […***…]. The reduction set forth in this Clause 9.9.5 shall be in addition to any reduction applied pursuant to Clause 9.9.3.

9.10 Escrow. If Customer's financial position materially improves at any time during the term of the Agreement, Customer may present Lonza with a credit risk re-assessment proposal, which Lonza will consider in good faith in assessing whether to release all or a portion of the escrowed funds.

10 Intellectual Property

10.1 Except as expressly otherwise provided herein, neither Party will, as a result of this Agreement, acquire any right, title, or interest in any Background Intellectual Property of the other Party.

10.2 Subject to Clause 10.3, Customer shall own all right, title, and interest in and to any and all Intellectual Property that Lonza and/or its Affiliates, the External Laboratories or other contractors or agents of Lonza develops, conceives, invents, first reduces to practice or makes, solely or jointly with Customer or others, in the course of performance of the Services that is principally a direct derivative of or improvement to, or that uses or incorporates, Customer Information and/or Customer Background Intellectual Property (collectively, the "New Customer Intellectual Property"). For avoidance of doubt, "New Customer Intellectual Property" shall include any material, processes or other items that solely embody, or that solely are claimed or covered by, any of the foregoing Intellectual Property, but shall exclude any New General Application Intellectual Property.

10.3 Notwithstanding Clause 10.2, and subject to the licenses granted in Clause 10.5, Lonza shall own all right, title and interest in Intellectual Property that Lonza and/or its Affiliates, the External Laboratories or other contractors or agents of Lonza, solely or jointly with Customer, develops, conceives, invents, or first reduces to practice or makes in the course of performance of the Services that (i) is generally applicable to the development or manufacture of chemical or biological products or product components or (ii) is an improvement of, or direct derivative of, any Lonza

Background Intellectual Property, and, in each case, does not require the use of, or incorporate any Customer Information and/or Customer Background Intellectual Property ("New General Application Intellectual Property"). For avoidance of doubt, "New General Application Intellectual Property" shall include any material, processes or other items that embody, or that are claimed or covered by, any of the foregoing Intellectual Property.

10.4 Lonza hereby assigns to Customer all of its right, title and interest in any New Customer Intellectual Property. Lonza shall execute, and shall require its personnel as well as its Affiliates, External Laboratories or other contractors or agents and their personnel involved in the performance of the Services to execute, any documents reasonably required to confirm Customer's ownership of the New Customer Intellectual Property, and any documents required to apply for, maintain and enforce any patent or other right in the New Customer Intellectual Property.

10.5 Subject to the terms and conditions set forth herein, Lonza hereby grants to Customer a non-exclusive, world-wide, fully paid-up, irrevocable, transferable license, including the right to grant sublicenses, under the New General Application Intellectual Property, to use, sell, have sold, offer for sale and import the Product manufactured under this Agreement. In addition, if Lonza incorporates any of its Background Intellectual Property (including the New General Application Intellectual Property described above) into the Manufacturing Process without first obtaining Customer's written consent, then, subject to Clause 10.7, Lonza will grant to Customer a non-exclusive, world-wide, fully paid-up, irrevocable, transferable license, including the right to grant sublicenses, under such Intellectual Property, to develop, make, have made, use, sell, have sold, offer for sale and import the Product.

10.6 Customer hereby grants Lonza the non-exclusive right to use the Customer Information, Customer Background Intellectual Property and New Customer Intellectual Property during the Term solely for the purpose of fulfilling its obligations under this Agreement.

10.7 Customer will have an irrevocable right, exercisable by written notice to Lonza, to transfer the Manufacturing Process to itself and/or any Third Party for the manufacture of the Product (but no other product); *provided, however,* to the extent such technology transfer is to a Third Party and includes Lonza Confidential Information, Lonza Background Intellectual Property or New General Application Intellectual Property, such transfer would be subject to the Third Party entering into a reasonable and appropriate confidentiality agreement with Lonza, and to the extent such Third Party is or will be engaged as a contract manufacturer for Customer or otherwise derives a significant amount of its business revenue from providing contract manufacturing services, such technology transfer would be subject to a reasonable licensing fee (a "Technology Fee") to be agreed upon by the Parties, subject to the following:

10.7.1 to the extent such Lonza Confidential Information, Lonza Background Intellectual Property or New General Application Intellectual Property was incorporated into the Manufacturing Process by Lonza without Customer's prior written consent, no Technology Fee shall be due;

10.7.2 if there has been a Persistent Supply Failure, or if such transfer of the Manufacturing Process is due to termination of this Agreement

for reasons other than uncured material breach of Customer under Clause 14.3.6, then no Technology Fee shall be due;

10.7.3 if a Technology Fee is applicable, the amount shall be negotiated in good faith […***…]; and

10.7.4 In the event of any disagreement between the Parties under this Clause 10.7 with respect to whether the Technology Fee is due or as to the amount of any such Technology Fee, the Parties shall within thirty (30) days of a Party's written request, identify and appoint a mutually agreed upon independent industry expert (the "Expert"). In such case, each Party will provide the Expert with the relevant information as well as a briefing document setting forth specific detailed reasons underlying such Party's position, and the Expert shall within an additional thirty (30) days following his appointment, make the determination in a writing stating his reasons for whether the Technology Fee is due or the amount of any such Technology Fee, as applicable, which determination shall be final and binding on the Parties. All costs associated with identifying and utilizing such Expert shall be borne equally by the Parties. Notwithstanding the foregoing in this Clause 10.7.4 and pending resolution of the disagreement, Lonza shall continue to conduct technology transfer of the Manufacturing Process to Customer and/or its designated Third Party.

For the avoidance of doubt, Customer may, in its sole discretion, elect not to have transferred or disclosed to it any Lonza Confidential Information, Lonza Background Intellectual Property and New General Application Intellectual Property, in which case Customer's sole payment obligation with respect to exercise of its technology transfer right under this Clause 10.7 shall be as set forth in Clause 10.8. A technology transfer pursuant to this Clause 10.7 will include […***…].

10.8 If Customer exercises its technology transfer right under Clause 10.7, Lonza shall provide reasonable technology transfer assistance services and access to necessary documentation to Customer as reasonably necessary to complete such technology transfer and enable Customer or its Third Party designee to replicate the Manufacturing Process as performed by Lonza, and Customer shall pay Lonza for such services (at Lonza's then-standard rates) and reimburse Lonza for reasonable out-of-pocket expenses incurred in providing such services and access.

11 Warranties

11.1 Lonza Representations and Warranties. Lonza warrants that:

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11.1.1 the Services shall be performed in accordance with all Applicable Laws;

11.1.2 except with respect to any development services and Engineering Batches, all Product Delivered by Lonza hereunder shall: (a) conform to the applicable Specifications in effect at the time of Release; (b) be manufactured and delivered in accordance with the Quality Agreement and cGMP; (c) not be adulterated within the meaning of the United States Food, Drug and Cosmetic Act, as amended, and any regulations promulgated thereunder (the ***"Act"***); and (d) be free and clear of any lien, encumbrance or other Third Party claim (collectively, the "Product Warranty");

11.1.3 it or its Affiliate holds all necessary permits, approvals, consents and licenses to enable it to perform the Services at the Facility;

11.1.4 it has not been debarred by the FDA pursuant to 21 U.S.C. § 335a or its successor provisions;

11.1.5 it will not use in the performance of Services hereunder, any personnel, Affiliate or Third Party that has been debarred by the FDA pursuant to 21 U.S.C. § 335a or its successor provisions; and

11.1.6 it has the necessary corporate authorizations to enter into and perform this Agreement.

11.2 Customer Representations and Warranties. Customer warrants that:

11.2.1 to Customer's knowledge as of the Effective Date, Customer has all the rights necessary to permit Lonza to manufacture Products without infringing the Intellectual Property rights of any Third Party and the manufacture of Product does not infringe any Third Party Intellectual Property rights;

11.2.2 Customer will promptly notify Lonza in writing if it receives or is notified of a formal written claim from a Third Party that Customer Information and/or Customer Intellectual Property or the use thereof by Lonza in the provision of the Services infringes any Intellectual Property or other rights of any Third Party; and

11.2.3 Customer has the necessary corporate authorizations to enter into this Agreement.

11.3 DISCLAIMER: THE WARRANTIES EXPRESSLY SET FORTH IN THIS AGREEMENT ARE IN LIEU OF ALL OTHER WARRANTIES, AND ALL OTHER WARRANTIES, BOTH EXPRESS AND IMPLIED, ARE EXPRESSLY DISCLAIMED, INCLUDING WITHOUT LIMITATION ANY WARRANTY OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE.

12 Indemnification and Liability

12.1 Indemnification by Lonza. Lonza shall indemnify, defend and hold harmless the Customer, its Affiliates, and their respective officers, employees and agents ("Customer Indemnitees") from and against any loss, damage, costs and expenses, including reasonable attorney fees (collectively, "Losses") that Customer Indemnitees

may suffer as a result of any Third Party claim to the extent such Losses arise out of or result from: (i) any material breach of this Agreement by Lonza; (ii) gross negligence or willful misconduct on the part of one or more of the Lonza Indemnitees (defined below); or (iii) any claims alleging that the Services (excluding use by Lonza of Customer Information and/or Customer Background Intellectual Property) infringe any Intellectual Property rights of a Third Party; except, in each case, to the extent that such claims resulted from the negligence, intentional misconduct or breach of this Agreement by any Customer Indemnitees.

12.2 Indemnification by Customer. Customer shall indemnify, defend and hold harmless Lonza, its Affiliates, and their respective officers, employees and agents ("Lonza Indemnitees") from and against any Losses that Lonza Indemnitees may suffer as a result of any Third Party claim to the extent such Losses arise out of or result from: (i) any material breach of this Agreement; (ii) gross negligence or willful misconduct on the part of one or more of the Customer Indemnitees; (iii) any claims alleging that the performance of Services (except to the extent the claim is based on use by Lonza of Lonza Information or Lonza Background Intellectual Property) infringes any Intellectual Property rights of third parties; or (iv) the manufacture, use, sale, or distribution of any Product, including any claims of product liability; except, in each case, to the extent that such claims resulted from the negligence, intentional misconduct or breach of this Agreement by any Lonza Indemnitees.

12.3 Indemnification Procedure. If the Party to be indemnified intends to claim indemnification under this Clause 12, it shall promptly notify the indemnifying Party in writing of such claim. The indemnitor shall have the right to control the defense and/or settlement thereof; provided, however, that any indemnitee shall have the right to retain its own counsel at its own expense. The indemnitee, its employees and agents, shall reasonably cooperate with the indemnitor in the investigation of any liability covered by this Clause 12. The failure to deliver prompt written notice to the indemnitor of any claim, to the extent prejudicial to its ability to defend such claim, shall relieve the indemnitor of any obligation to the indemnitee under this Clause 12.

12.4 DISCLAIMER OF CONSEQUENTIAL DAMAGES. Except in the case of a Party's breach of Clause 13, IN NO EVENT SHALL EITHER PARTY BE LIABLE TO THE OTHER PARTY FOR INCIDENTAL, INDIRECT, SPECIAL, PUNITIVE OR CONSEQUENTIAL DAMAGES, LOST PROFITS OR LOST REVENUES ARISING FROM OR RELATED TO THIS AGREEMENT, EXCEPT TO THE EXTENT RESULTING FROM FRAUD, GROSS NEGLIGENCE OR INTENTIONAL MISCONDUCT BY SUCH PARTY; *provided, however,* that this Clause 12.4 shall not be construed to limit the Parties' indemnification obligations with respect to Third Party claims under Clauses 12.1 and 12.2.

12.5 LIMITATION OF LIABILITY. LONZA'S LIABILITY UNDER THIS AGREEMENT FOR CLAIMS RELATING TO LONZA'S BREACH OF ITS OBLIGATIONS WITH RESPECT TO COMPLETION OF THE DETAILED DESIGN OR OTHER OBLIGATIONS RELATING TO DESIGN OR CONSTRUCTION OF THE FACILITY PRIOR TO EXERCISE OF THE CONSTRUCTION TRIGGER, SHALL IN NO EVENT EXCEED, IN THE AGGREGATE FOR ALL SUCH CLAIMS, […***…]. LONZA'S LIABILITY UNDER THIS AGREEMENT FOR CLAIMS RELATING TO CONSTRUCTION OF THE FACILITY SHALL IN NO EVENT EXCEED, IN THE AGGREGATE FOR ALL SUCH CLAIMS, THE TOTAL AMOUNT OF […***…] TO LONZA IN THE EVENT OF EARLY TERMINATION. LONZA'S LIABILITY UNDER THIS AGREEMENT FOR ALL OTHER CLAIMS SHALL IN NO EVENT EXCEED, IN

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THE AGGREGATE FOR CLAIMS BASED ON SIMILAR OR RELATED EVENTS, […*…] ([…***…]) MONTH PERIOD PRECEDING THE FIRST SUCH EVENT. NOTWITHSTANDING THE FOREGOING, IN NO EVENT SHALL THE LIMITATIONS ON LIABILITY SET FORTH IN THIS CLAUSE 12.5 APPLY TO ANY DAMAGES TO THE EXTENT RESULTING FROM LONZA'S […***…] OR […***…].**

13 Confidentiality

13.1 A Party receiving Confidential Information (the "Receiving Party") agrees to strictly keep secret any and all Confidential Information received during the Term from or on behalf of the other Party (the "Disclosing Party") using at least the same level of measures as it uses to protect its own Confidential Information, but in any case at least commercially reasonable and customary efforts. For purposes of this Clause 13, New Customer Intellectual Property shall be treated as Confidential Information of Customer, and Customer and Lonza shall be deemed the Disclosing Party and Receiving Party, respectively, with respect thereto, regardless of which party first discloses New Customer Intellectual Property. Confidential Information shall include information disclosed in any form including but not limited to in writing, orally, graphically or in electronic or other form to the Receiving Party, observed by the Receiving Party or its employees, agents, consultants, or representatives, or otherwise learned by the Receiving Party under this Agreement, which the Receiving Party knows or reasonably should know is confidential or proprietary. This Agreement and its terms shall be Confidential Information of each Party.

13.2 Notwithstanding the foregoing, Receiving Party may disclose to any courts and/or other authorities Confidential Information which is or will be required pursuant to applicable governmental or administrative or public law, rule, regulation or order. In such case the Party that received the Confidential Information will, to the extent legally permitted, inform the other Party promptly in writing and cooperate with the Disclosing Party in seeking to minimize the extent of Confidential Information which is required to be disclosed to the courts and/or authorities.

13.3 The obligation to maintain confidentiality under this Agreement does not apply to Confidential Information, which:

13.3.1 at the time of disclosure was publicly available; or

13.3.2 is or becomes publicly available other than as a result of a breach of this Agreement by the Receiving Party; or

13.3.3 as the Receiving Party can establish by competent proof, was rightfully in its possession at the time of disclosure by the Disclosing Party and had not been received from or on behalf of Disclosing Party (provided, that New Customer Intellectual Property shall not be subject to this Clause 13.3.3); or

13.3.4 is supplied to a Party by a Third Party which was not in breach of an obligation of confidentiality to Disclosing Party or any other party; or

13.3.5 is developed by the Receiving Party independently from and without use of the Confidential Information, as evidenced by contemporaneous written records.

13.4 The Receiving Party will use Confidential Information only for the purposes of this Agreement and will not make any use of the Confidential Information except as necessary to perform its obligations or to exercise its rights under this Agreement. The Receiving Party agrees to return or destroy promptly (and certify such destruction) on Disclosing Party's request all written or tangible Confidential Information of the Disclosing Party, except that one copy of such Confidential Information may be kept by the Receiving Party in its confidential files for record keeping purposes only.

Each Party will restrict the disclosure of Confidential Information to such officers, employees, consultants and representatives of itself and its Affiliates who have been informed of the confidential nature of the Confidential Information and who have a need to know such Confidential Information for the purpose of this Agreement. Prior to disclosure to such persons, the Receiving Party shall bind its and its Affiliates' officers, employees, consultants and representatives to confidentiality and non-use obligations no less stringent than those set forth herein. The Receiving Party shall notify the Disclosing Party as promptly as practicable of any unauthorized use or disclosure of the Confidential Information. Additionally, each Party shall have the right to disclose Confidential Information of the other Party (including the Agreement) to actual or potential Third Party investors or actual or potential Third Party acquirers, licensees or collaborative or other partners, and to their bankers, lawyers, accountants, agents, provided, in each case that each such Third Party or advisor thereof is bound to confidentiality and non-use obligations no less stringent than those set forth herein.

13.5 The Receiving Party shall at any time be fully liable for any and all breaches of the confidentiality obligations in this Clause 13 by any of its Affiliates or the employees, consultants and representatives of itself or its Affiliates.

13.6 Each Party hereto expressly agrees that any breach or threatened breach of the undertakings of confidentiality provided under this Clause 13 by a Party may cause irreparable harm to the other Party and that money damages may not provide a sufficient remedy to the non-breaching Party for any breach or threatened breach. In the event of any breach and/or threatened breach, then, in addition to all other remedies available at law or in equity, the non-breaching Party shall be entitled to seek injunctive relief and any other relief deemed appropriate by the non-breaching Party.

13.7 The Parties shall coordinate in advance with each other in connection with the disclosure or filing of this Agreement (including redaction of certain provisions of this Agreement) with the U.S. Securities and Exchange Commission (the "SEC"), the NASDAQ stock exchange or any other stock exchange or governmental agency on which securities issued by a Party or its Affiliate are traded, and each Party shall use reasonable efforts to seek confidential treatment for the terms proposed to be redacted; provided, that each Party shall ultimately retain control over what information to disclose to the SEC, the NASDAQ stock exchange or any other stock exchange or governmental agency, as the case may be. Other than such obligation, neither Party (nor its Affiliates) shall be obligated to consult with or obtain approval from the other Party with respect to any filings to the SEC, the NASDAQ stock exchange or any other stock exchange or governmental agency.

14 Term and Termination

14.1 Term. This Agreement shall commence on the Effective Date and shall end on the earlier of (a) the sixth (6th) anniversary of the date of First Approval, or (b) expiration of the Reservation Period prior to Customer exercising the Construction Trigger, unless terminated earlier as provided herein or extended as provided herein (the "Term"). Notwithstanding the foregoing, each Project Plan may have separate term and termination provisions so long as the term of any Project Plan does not extend beyond the Term.

14.2 Extension. If Customer exercises the Construction Trigger, begins paying the Monthly […***…] Fee (as defined below) prior to the third anniversary of First Approval, and thereafter continues paying the Monthly […***…] Fee (or a higher monthly suite fee) until the end of the initial term, then Customer shall have a one-time extension right, exercisable by written notice prior to the date that is four years following First Approval and subject to continued payment of the Monthly […***…] Fee (or a higher monthly suite fee), to extend the term of the Agreement for an additional three years (for a total term of nine years following First Approval). Such extension would be subject to a good faith negotiation of commercially reasonable pricing applicable during the extension term, provided that the lowest monthly suite fee during any extension term would not be lower than the Monthly […***…] Fee. Any further extension of the term would be subject to mutual agreement. At either party's request, the parties will engage in good faith negotiations regarding such further extension during the […***…]-year period prior to expiration, provided neither party shall be obligated to further extend the term unless and until a definitive agreement regarding extension is executed and delivered by the parties.

14.3 Termination. This Agreement may be terminated as follows:

14.3.1 Customer shall have the right to terminate this Agreement at any time during the Reservation Period upon written notice to Lonza, provided, that upon exercise of the Construction Trigger, Customer's right to terminate this Agreement pursuant to this sub-clause shall terminate and be of no further force or effect.

14.3.2 After exercise of the Construction Trigger but prior to completion of the first Engineering Batch, Customer shall have the right to terminate this Agreement upon ninety (90) days' written notice to Lonza in the event that: (a) Customer receives from FDA (i) notice of FDA's refusal to approve Customer's application for approval to market the Product or (ii) a complete response letter indicating that Customer's application for approval to market the Product cannot be approved without one or more additional clinical trials being performed (in each case, a "Product Non-Approval Notice"), and a Customer Withdrawal occurs; (b) Customer receives from FDA notice of FDA's withdrawal of approval to market the Product ("FDA Withdrawal Notice"), and a Customer Withdrawal occurs; or (c) a Customer Withdrawal occurs.

14.3.3 After completion of first Engineering Batch but prior to First Approval, Customer shall have the right to terminate this Agreement upon ninety (90) days' written notice to Lonza in the event that (a) Customer receives a Product Non-Approval Notice and a Customer Withdrawal subsequently occurs; or (b) a Customer Withdrawal occurs.

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14.3.4 After First Approval, Customer shall have the right to terminate this Agreement upon ninety (90) days' written notice to Lonza in the event that (a) marketing approval of the Product is withdrawn by the FDA and the EMA (to the extent such marketing approval had previously been granted in each such jurisdiction) and a Customer Withdrawal subsequently occurs; or (b) a Customer Withdrawal occurs.

14.3.5 Customer shall have the right to terminate immediately upon written notice to Lonza upon the occurrence of any of the following events: (a) failure of Lonza to timely perform its obligations under Clause 3.2.1 and to cure such failure within […***…] ([…***…]) days of receiving written notice of such failure from Customer; (b) a […***…]; or (c) Lonza, any of its Affiliates or Third Party subcontractors performing Services hereunder, or any person used by any of them to perform Services hereunder, or any of their respective officers or directors, as applicable, involved in performing Services, is debarred by the FDA, under the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 335(a), (b)(1), and (b)(2)) and this adversely impacts Lonza's ability to perform the Services hereunder (ie., Lonza is unable to replace such Affiliate, Third Party subcontractor or person in a timely manner).

14.3.6 by either Party if the other Party breaches a material provision of this Agreement or a Project Plan and fails to cure such breach to the reasonable satisfaction of the non-breaching Party within ninety (90) days (except that the cure period for non-payment shall be ten (10) days and the cure period for Lonza's breach of Clause 3.2.1 shall be thirty (30) days) following written notification of such breach from the non-breaching party to the breaching party; provided, however, that such ninety (90) day period shall be extended as agreed by the Parties if the identified breach is incapable of cure within ninety (90) days and if the breaching Party provides a plan and timeline to cure the breach, promptly commences efforts to cure the breach and diligently prosecutes such cure; and provided, further, that this extended period shall be unavailable for (a) any breach regarding non-payment, or (b) any breach by Lonza of Clause 3.2.1;

14.3.7 by either Party, immediately, if the other Party becomes insolvent, is dissolved or liquidated, makes a general assignment for the benefit of its creditors, or files or has filed against it, a petition in bankruptcy or has a receiver appointed for a substantial part of its assets; or

14.3.8 by either Party pursuant to Clause 15.

14.4 Consequences of Termination. In the event of termination hereunder, Lonza shall be compensated for (i) […***…]; and (ii) all costs incurred through the date of termination, including Raw Materials costs and Raw Materials Fees for Raw Materials used or purchased for use in connection with the Project Plan (but without […***…] and excluding, in each case, […***…]). In addition:

14.4.1 Upon termination pursuant to Clause 14.3.2, Customer shall pay to Lonza […***…] ([…***…] date of termination) plus an amount equal to […***…] ([…***…]) […***…] of […***…] at the […***…]. If notice of termination occurs during a month prior to a month when the […***…] would be due then the […***…].

14.4.2 Upon termination pursuant to Clause 14.3.3 or 14.3.4, Customer shall pay to Lonza […***…] ([…***…] prior to the date of termination) plus an amount equal to […***…] ([…***…]) […***…] at the rate paid during the month of termination notification.

14.4.3 In the event of Lonza's […***…], or in the event of Customer's termination pursuant to Clause 14.3.5 or 14.3.6, then (a) until product is approved at a new manufacturing site, Lonza will make available Lonza employees to Customer at the new manufacturing site to the extent necessary, at Customer's cost and expense, to provide reasonable technical support and assistance, for a duration of time not to exceed […***…] months; and (b) Lonza shall provide the assistance described in Clause 10.8 at no charge to Customer.

14.4.4 In the event of any such termination pursuant to Clause 14.3.5 or 14.3.6, all amounts remaining in the Escrow Account shall be returned to Customer pursuant to Clause 14.4.5.

14.4.5 Upon termination of this Agreement and payment in full of all amounts owed under this Clause 14.4 by Customer, the Parties will cooperate to send a joint notice to the Escrow Agent terminating the Escrow Agreement and instructing the Escrow Agent to return any amounts remaining in the Escrow Account to Customer.

14.5 Survival. The rights and obligations of each Party which by their nature survive the termination or expiration of this Agreement shall survive the termination or expiration of this Agreement, including Clauses 1, 6, 10, 12-14, 15.1 and 16 (to the extent relevant).

15 Force Majeure

15.1 If Lonza is prevented or delayed in the performance of any of its obligations under the Agreement by Force Majeure and gives written notice thereof to Customer specifying the matters constituting Force Majeure together with such evidence as Lonza reasonably can give and specifying the period for which it is estimated that such prevention or delay will continue, Lonza shall be excused from the performance or the punctual performance of such obligations as the case may be from the date of such notice for so long as such cause of prevention or delay shall continue. If a Force Majeure persists for more than […***…] ([…***…]) days, Customer shall then be entitled to a reduced facility fee rate equal to half of the then current rate. If such Force Majeure persists for an additional […***…] days, then Customer shall have the right to terminate the Agreement upon written notice to Lonza and, if Customer had

previously exercised the Construction Trigger and subject to Clause 6.2 (if applicable), […***…] ([…***…]).

15.2 "Force Majeure" shall mean any reason or cause beyond Lonza's reasonable control affecting the performance by Lonza of its obligations under the Agreement, including, but not limited to, any cause arising from or attributable to acts of God, strike, lockouts, restrictive governmental orders or decrees, riots, insurrection, war, terrorists acts, interruption of energy supplies or the inability of Lonza to obtain any required raw material, equipment or transportation due to a general shortage of such materials, equipment or transportation.

15.3 With regard to Lonza, any such event of Force Majeure affecting services or production at its Affiliates or suppliers shall be regarded as an event of Force Majeure.

16 Miscellaneous

16.1 Non-Solicitation. During the term of this Agreement and for […***…] ([…***…]) years thereafter, each of the Parties agrees not to seek to induce or solicit any employee of the other Party or its Affiliates to discontinue his or her employment with the other Party or its Affiliate in order to become an employee or an independent contractor of the soliciting Party or its Affiliate; *provided, however,* that neither Party shall be in violation of this Clause 16.1 as a result of making a general solicitation for employees or independent contractors. For the avoidance of doubt, the publication of an advertisement shall not constitute solicitation or inducement.

16.2 Severability. If any part of this Agreement shall be found to be invalid or unenforceable under applicable law in any jurisdiction, such part shall be ineffective only to the extent of such invalidity or unenforceability in such jurisdiction, without in any way affecting the remaining parts of this Agreement in that jurisdiction or the validity or enforceability of the Agreement as a whole in any other jurisdiction. The Parties hereto shall use their commercially reasonable efforts to replace the invalid, illegal or unenforceable provision(s) with valid, legal and enforceable provision(s) in a way that, to the extent practicable and legally permissible, implements the original intent of the Parties.

16.3 Amendments. Modifications and/or amendments of this Agreement must be in writing and signed by the Parties.

16.4 Assignment. Neither Party may assign its interest under the Agreement without the prior written consent of the other Party, such consent not to be unreasonably withheld; *provided, however,* that either Party may assign this Agreement: (i) to any Affiliate of such Party, provided that the assigning Party shall remain liable and responsible to the non-assigning Party hereto for the performance and observance of all such duties and obligations by such Affiliate; or (ii) in connection with the transfer or sale of all or substantially all of the business of such Party to which this Agreement relates to a Third Party, whether by merger, sale of stock, sale of assets or otherwise (a "Sale Transaction"), provided, in the case of a Sale Transaction involving Customer, that the acquiring Third Party in such Sale Transaction is not an entity whose business primarily derives from providing contract manufacturing services. In addition, Lonza shall be entitled to sell, assign and/or transfer its trade receivables resulting from this Agreement without the consent of the Customer. The rights and obligations of the Parties under this Agreement shall be binding upon and inure to the benefit of the successors and permitted assigns of the Parties, and the name of a

Party appearing herein will be deemed to include the name of such Party's successors and permitted assigns to the extent necessary to carry out the intent of this clause. Any purported assignment of this Agreement not in accordance with this Clause 16.4 shall be void. No assignment shall relieve any Party of responsibility for the performance of any obligation that accrued prior to the effective date of such assignment.

16.5 Notice. All notices required or permitted hereunder shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified; (b) when sent by confirmed facsimile or email if sent during normal business hours of the recipient, and if sent other than during normal business hours of the recipient, on the next business day; (c) five calendar days after having been sent by registered or certified mail, return receipt requested, postage prepaid; or (d) one business day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent to the other party hereto at such party's mailing address, facsimile number or email address set forth below, or at such other mailing address, facsimile number or email address as such party may designate by 10 days' advance written notice to the other party hereto.

If to Lonza:

Lonza Biologics, Inc.
101 International Drive
Portsmouth, New Hampshire 03801
Attention: Carson Sublett, Sr. Site Director
Fax:
Email: […***…]

With a copy to:

Lonza America Inc.
90 Boroline Road
Allendale, New Jersey 07401
Attention: Scott Waldman, General Counsel
Fax:
Email: […***…]

If to Customer:

Celladon Corporation
11988 El Camino Real, Suite 650
San Diego, CA 92130-3579
USA
Attention: Paul Cleveland, President and CFO
Fax: […***…]
Email: […***…]

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With a copy to:

Celladon Corporation
11988 El Camino Real, Suite 650
San Diego, CA 92130-3579
USA
Attention: Elizabeth Reed, Vice President and General Counsel
Fax: […***…]
Email: […***…]

16.6 Governing Law. This Agreement is governed in all respects by the laws of Delaware without regard to its conflicts of laws principles.

16.7 Entire Agreement. This Agreement (including the Appendices hereto) and the Clinical Agreement (as it relates to the statements of work entered into by the Parties thereunder) collectively contain the entire agreement between the Parties as to the subject matter hereof and thereof and supersede all prior and contemporaneous agreements with respect to the subject matter hereof and thereof. This Agreement may be executed in any number of counterparts, each of which shall be deemed to be an original, and all of which together shall constitute one and the same document. Each party acknowledges that an original signature or a copy thereof transmitted by facsimile or by .pdf shall constitute an original signature for purposes of this Agreement.

16.8 Independent Contractors. Each of the Parties is an independent contractor and nothing herein contained shall be deemed to constitute the relationship of partners, joint venturers, nor of principal and agent between the Parties. Neither Party shall at any time enter into, incur, or hold itself out to Third Parties as having authority to enter into or incur, on behalf of the other Party, any commitment, expense, or liability whatsoever.

16.9 Dispute Resolution. Other than disputes under Clause 4.3, if the Parties are unable to resolve a dispute despite their good faith efforts, either Party may refer the dispute to the President or Head of each Party's respective business unit (or other designee). In the event that no agreement is reached by such representatives with respect to such dispute within thirty (30) days after its referral to them, either Party may pursue any and all remedies available at law or in equity.

16.10 Waiver. The failure of any Party at any time or times to require performance of any provision of this Agreement or any Project Plan will in no manner affect its rights at a later time to enforce the same. No waiver by any Party of any term, provision or condition contained in this Agreement or any Project Plan, whether by conduct or otherwise, in any one or more instances, shall be deemed to be or construed as a further or continuing waiver of any such term, provision or condition or of any other term, provision or condition of this Agreement or any Project Plan.

16.11 Titles and Subtitles. All headings, titles and subtitles used in this Agreement or any Project Plan are for convenience only and are not to be considered in construing or interpreting any term or provision of this Agreement or any Project Plan.

16.12 Pronouns. Where the context requires, (i) all pronouns used herein will be deemed to refer to the masculine, feminine or neuter gender as the context requires, and (ii) the singular context will include the plural and vice versa.

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16.13 No Presumption Against Drafter. For purposes of this Agreement, each Party hereby waives any rule of construction that requires that ambiguities in this Agreement or any Project Plan be construed against the drafter.

IN WITNESS WHEREOF, each of the Parties hereto has caused this Facility Construction and Commercial Supply Agreement to be executed by its duly authorized representative effective as of the date written above.

LONZA BIOLOGICS, INC.

By: /s/ Scott Waldman
Name: Scott Waldman
Title: President

CELLADON CORPORATION

By: /s/ Paul B. Cleveland
Name: Paul B. Cleveland
Title: President and Chief Financial Officer

Appendix A
Project Plans

[…***…]

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Appendix C
Estimated Construction Timeline

[…***…]

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Appendix D
Minimum Production Targets

[…***…]

[…***…]

[...***...] [...***...]

[…***…]

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Appendix E
Form of Escrow Agreement

[…***…]

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Appendix G
Form of Common Stock Purchase Agreement

[…***…]

***Confidential Treatment Requested

Appendix H
Customer Background Intellectual Property

[…***…]

***Confidential Treatment Requested

Exhibit 10.35

*****Text Omitted and Filed Separately with the Securities and Exchange Commission. Confidential Treatment Requested Under 17 C.F.R. Sections 200.80(b)(4) and 240.24b-2.**

DEVELOPMENT, MANUFACTURING AND SUPPLY AGREEMENT

THIS DEVELOPMENT, MANUFACTURING AND SUPPLY AGREEMENT (***"Agreement"***) is made and entered into as of March 20, 2015 (the ***"Effective Date"***), by and between **CELLADON CORPORATION**, a Delaware corporation with offices at 11988 El Camino Real, Suite 650, San Diego, CA 92130-3579, USA (***"Celladon"***), and **NOVASEP, INC.**, a New Jersey corporation having offices at 23 Creek Circle, Boothwyn, PA 19061, USA (***"Novasep"***).

RECITALS

WHEREAS, Celladon is engaged in the development of its proprietary AAV1/SERCA2a gene therapy candidate known as MYDICAR® (***"Mydicar"***);

WHEREAS, Novasep, through its affiliate Henogen (as defined below), provides contract manufacturing services to the biopharmaceutical industry, including process and analytical transfer, downstream and upstream process development, process scale-up and validation, and cGMP manufacturing services with respect to viruses and viral vectors for use in pharmaceutical products;

WHEREAS, Celladon and Novasep are parties to that certain letter agreement dated as of December 19, 2014 (the ***"Letter Agreement"***);

WHEREAS, as described in the Letter Agreement, Celladon and Novasep wish to enter into a collaborative relationship related to Celladon's proprietary AAV1-based vector containing the expression cassette for SERCA2a, the active pharmaceutical ingredient of Mydicar, pursuant to which (i) the parties would implement a transfer program to enable Novasep to produce bulk drug substance of such active pharmaceutical ingredient on behalf of Celladon for use in the production of Mydicar for commercial distribution, (ii) Novasep would make facility modifications necessary for the manufacture of such bulk drug substance and perform process development, scale-up and validation services necessary for commercial production of such bulk drug substance, and (iii) Novasep would manufacture and supply such bulk drug substance to Celladon for use in the production of Mydicar for commercial distribution (collectively, the ***"Project"***);

WHEREAS, pursuant to the Letter Agreement, Celladon and Novasep agreed to certain binding rights and obligations with respect to preliminary Project activities that the parties wished to commence, or reserve capacity for (as applicable), prior to the negotiation and execution by the Celladon and Novasep of a definitive agreement governing the Project; and

WHEREAS, the parties now wish to enter into this Agreement to govern the relationship between the parties and to define the conditions under which Novasep will perform the development and manufacturing services described above with respect to Product (defined below); in each case, on the terms and subject to the conditions set forth in this Agreement.

NOW, THEREFORE, in consideration of the foregoing premises and the mutual covenants contained herein and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Celladon and Novasep hereby agree as follows:

1. DEFINITIONS

1.1 "Acceptance Period" shall have the meaning set forth in Section 5.2(a).

1.2 "Additional Amount" shall have the meaning set forth in Section 7.1.

1.3 "Affiliate" shall mean, with respect to a company or other business entity (including a party hereto), any other company or business entity controlled by, controlling, or under common control with such company or other business entity. For the purposes of this definition, the term "control" (including, with correlative meanings, the terms "controlled by" and "under common control with") shall mean the possession, directly or indirectly, of more than 50% of the outstanding voting securities of a corporation or comparable equity interest in any other type of entity, or otherwise having the power to direct the management and policies of such corporation or other entity.

1.4 "Applicable Law" shall mean any applicable laws, rules and regulations, including any rules, regulations, guidelines or other requirements of relevant government agencies, that may be in effect from time to time in the applicable country or jurisdiction, and that may apply to the Development Services and/or the manufacture and supply of the Product under this Agreement.

1.5 "Batch" shall mean […***…].

1.6 "Batch Documentation" shall have the meaning set forth in Section 5.1.

1.7 "Batch Price" shall mean, with respect to a particular cGMP […***…] to be supplied by Novasep to Celladon hereunder, the amount that Celladon shall pay Novasep for supply of such […***…] to Celladon as set forth in Section 7.2 and **Exhibit A** hereto, subject to adjustment in accordance with Section 7.3.

1.8 "Batch Records" shall mean, with respect to a particular production run conducted by Novasep for manufacturing one Batch of Product, the completed, executed batch records, in the form of the Master Batch Records, containing all the relevant manufacturing and in-process and batch release testing details and information for such production run, including any deviations and out of specification results. The contents of the Batch Records shall be as described in the Quality Agreement.

1.9 "Batch Sample" shall have the meaning set forth in Section 5.1.

1.10 "Binding Period" shall have the meaning provided in Section 4.5(b).

1.11 "Business Day" shall mean any day other than a Saturday, a Sunday or any public holiday in Seneffe, Belgium, or San Diego, California, USA.

1.12 "Celladon Background IP" shall mean any and all: (a) Information that: (i) is either (A) Controlled by Celladon as of the Effective Date or (B) developed or acquired by or on behalf of, and Controlled by, Celladon independently from the activities contemplated by this

Agreement during the Term; (ii) relates to, and is necessary or reasonably useful for, the manufacture of Product; and (iii) is proprietary to, or is maintained in confidence by, Celladon; including, without limitation, all Celladon Materials; and (b) patent and other intellectual property rights Controlled by Celladon that claim, cover or are appurtenant to any of the foregoing; but excluding, in each case, Celladon Initial Project IP.

1.13 "Celladon Initial Project IP" shall have the meaning set forth in Section 1.16(a).

1.14 "Celladon Materials" shall mean any and all cell lines, cell banks, virus seed, viruses, viral vectors, reagents, reference standards and/or other materials that Celladon may deem necessary to be transferred to Novasep, free of charge, in order for Novasep to manufacture Product, including, without limitation, […***…].

1.15 "Celladon Materials Specifications" shall mean the Celladon Materials' attributes, characteristics, tests performed, acceptance criteria, storage and packaging requirements to be mutually agreed by the parties before commencement of the production run for the first Engineering Batch and to be attached hereto as **Exhibit B** (which will be incorporated herein by this reference), as the same may be amended or supplemented from time to time in accordance with this Agreement.

1.16 "Celladon Materials Warranty" shall have the meaning set forth in Section 9.2(a).

1.17 "Celladon New Project IP" shall have the meaning set forth in Section 1.16(b).

1.18 "Celladon Project IP" shall mean any and all:

(a) (i) Information, inventions, developments and discoveries (whether or not patentable) developed, conceived, invented, first reduced to practice, made or generated by or on behalf of Novasep, any of its Affiliates, or any of Novasep's or its Affiliates' respective subcontractors (either solely or jointly with Celladon or others), in the course of performance of the Initial Project Plan (as defined in the Letter Agreement) on or after the Collaboration Initiation Date and prior to the Effective Date that constitute a modification or improvement of, or that use or incorporate, Celladon's Confidential Information; and (ii) patent and other intellectual property rights in or to any of the foregoing (collectively, ***"Celladon Initial Project IP"***); and

(b) (i) Information, inventions, developments and discoveries (whether or not patentable) developed, conceived, invented, first reduced to practice, made or generated by or on behalf of Novasep, any of its Affiliates, or any of Novasep's or its Affiliates' respective subcontractors (either solely or jointly with Celladon or others), in the course of performance of the Services on or after the Effective Date that (A) […***…], or (B) otherwise […***…]; including, in each case, any of the foregoing that […***…]

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[…***…]; and (ii) patent and other intellectual property rights in or to any of the foregoing (collectively, ***"Celladon New Project IP"***).

1.19 "Celladon Technology" shall mean Celladon Background IP and Celladon Project IP.

1.20 "cGMP" shall mean the current standards for the manufacture of pharmaceutical products, pursuant to (a) the FD&C Act (21 U.S.C. 321 et seq.); (b) relevant United States regulations in Title 21 of the United States Code of Federal Regulations (including Parts 11, 210, 211, 600 and 610); (c) EC Directive 2003/94/EC of October 8, 2003; (d) the EC Guide to Good Manufacturing Practice for Medicinal Products Part II; (e) International Conference on Harmonization (ICH) ICH Q7 Good Manufacturing Practice Guide for Active Pharmaceutical Ingredients; and (f) all additional Regulatory Authority documents or regulations that replace, amend, modify, supplement or complement any of the foregoing.

1.21 "cGMP Batch" shall mean a Batch that is required under the Scope of Work, the applicable Project Plan or a Purchase Order to be manufactured in compliance with cGMP.

1.22 "CMC" shall mean chemistry, manufacturing and controls.

1.23 "Collaboration Initiation Date" shall mean December 19, 2014.

1.24 "Commercially Reasonable Efforts" means, as it relates to either Novasep or Celladon hereunder, the expenditure by such party of the efforts and resources with respect to a particular task or set of activities in a manner […***…] stage in development and life cycle.

1.25 "Confidential Information" of a party shall mean, subject to the exceptions set forth in Section 10.2, any and all Information that was or is disclosed, transferred or made available by or on behalf of such party (the ***"Disclosing Party"***) to the other party (the ***"Receiving Party"***) pursuant to or in connection with the Letter Agreement (including the Initial Project Plan) or this Agreement, whether in writing, orally, visually or otherwise. For the avoidance of doubt, and without limiting the generality of the foregoing, the Manufacturing Process, the Specifications and the Celladon Materials are Confidential Information of Celladon. In addition, for purposes of this Agreement, "Confidential Information" (as defined in the Confidentiality Agreement) disclosed or transferred by Celladon to Groupe Novasep SAS pursuant to the Confidentiality Agreement shall be considered Confidential Information of Celladon, and "Confidential Information" (as defined in the Confidentiality Agreement) disclosed or transferred by Groupe Novasep SAS to Celladon pursuant to the Confidentiality Agreement shall be considered Confidential Information of Novasep. For the avoidance of doubt, and without limiting the generality of the foregoing, the Novasep Background IP is Confidential Information of Novasep and the Celladon Background IP is Confidential Information of Celladon, even if any patents or patent applications covering the foregoing become published or otherwise are in the public domain. Notwithstanding the foregoing, the

parties agree that all Celladon Project IP shall be considered the Confidential Information of Celladon, and Celladon shall be considered the "Disclosing Party" and Novasep shall be considered the "Receiving Party" with respect thereto.

1.26 "Confidentiality Agreement" shall mean the Mutual Confidential Disclosure Agreement between Celladon and Groupe Novasep SAS dated August 15, 2011, as amended by the Letter Agreement.

1.27 "Control" or **"Controlled"** shall mean, with respect to any Information, material, or patent or other intellectual property rights, possession by an entity of the ability (whether by ownership, license or otherwise) to grant access to, to grant use of, or to grant a license or a sublicense of or under such Information, material, or patent or other intellectual property rights without violating or conflicting with any agreement with or rights of a Third Party.

1.28 "Development Services" shall have the meaning set forth in Section 7.1.

1.29 "Disclosing Party" shall have the meaning set forth in Section 1.24.

1.30 "EMA" shall mean the European Medicines Agency, or any successor thereto having the administrative authority to regulate the development and marketing of human pharmaceutical products in the European Union.

1.31 "Engineering Batch" shall mean a Batch that is intended to demonstrate the transfer of the Manufacturing Process to the Novasep Facility.

1.32 "Engineering Batch Specifications" shall mean the specifications that will be applicable to the Engineering Batches to be produced by Novasep under this Agreement.

1.33 "FDA" shall mean the United States Food and Drug Administration, or any successor thereto having the administrative authority to regulate the development and marketing of human pharmaceutical products in the United States.

1.34 "FD&C Act" shall mean the United States Food, Drug and Cosmetic Act, as amended, and any regulations promulgated thereunder.

1.35 "Final Product" shall mean Mydicar, in finished form, containing Product supplied by Novasep hereunder.

1.36 "First Extension Option" shall have the meaning set forth in Section 11.1(a).

1.37 "Henogen" shall mean Novasep's Belgian Affiliate who owns the Novasep Development Site and the Novasep Facility and who will primarily perform the Development Services and manufacture the Product under this Agreement.

1.38 "Information" shall mean any and all (a) information, results and data, including discoveries, improvements, processes, methods, protocols, formulas, techniques, inventions, know-how and trade secrets, scientific, chemical, pharmaceutical, toxicological, biochemical, and biological, data, and information relating to the results of tests, assays, methods, processes,

and specifications, and/or other documents containing information and related data, and any assay control, regulatory, and any other test results or information, regulatory, manufacturing, financial and commercial information or data, and (b) compositions of matter, cells, cell lines, viruses, viral vectors, and other physical, biological or chemical materials.

1.39 "Initial Forecast" shall have the meaning set forth in Section 4.5.

1.40 "Initial Term" shall have the meaning set forth in Section 11.1.

1.41 "Letter Agreement" shall have the meaning set forth in the recitals to this Agreement.

1.42 "Licensee" shall mean any Third Party to which Celladon or its Affiliate grants a license to make, have made, use, sell, have sold, offer for sale or import Product.

1.43 "Manufacturing Process" shall mean the production process for the manufacture of Product as defined in the Master Batch Records.

1.44 "Manufacturing Schedule" shall have the meaning set forth in Section 4.3.

1.45 "Manufacturing SOPs" shall mean the specific methods, techniques, processes and standard operating procedures that are to be used by Novasep to manufacture Product, including the applicable Quality Control Procedures.

1.46 "Master Batch Records" shall mean the master or unexecuted batch records for Product as established by mutual written agreement of the parties, including the applicable Manufacturing SOPs, the in-process testing and QA/QC testing for such Product, which are to be used in the manufacture and testing of Product by Novasep hereunder.

1.47 "Maximum Capacity" shall mean the total number of Batches of Product that can be produced in one calendar year at the Modified Novasep Facility, with the Modified Novasep Facility operating […***…] hours per day and for […***…] days in the year. Starting in 2017, the Maximum Capacity for the Modified Novasep Facility will be determined, by extrapolating to a full calendar year the actual manufacturing cycle times experienced by Novasep in the Modified Novasep Facility for Product during the most recent manufacturing campaign, after the first half of the campaign, and if applicable the days required for preventive maintenance at the Novasep Facility. As of the Effective Date, the parties believe that the "Maximum Capacity" of the Modified Novasep Facility will be […***…] Batches in one calendar year.

1.48 "Modified Novasep Facility" shall mean the Novasep facility as modified by the Novasep Facility Modifications.

1.49 "Mydicar" shall have the meaning set forth in the recitals to this Agreement.

1.50 "Non-Conforming Batch" shall have the meaning set forth in Section 5.2(c).

1.51 "Novasep Background IP" shall mean any and all: (a) Information that: (i) is either (A) Controlled by Novasep as of the Effective Date or (B) developed or acquired by or on

behalf of, and Controlled by, Novasep independently from the activities contemplated by the Letter Agreement or this Agreement during the Term; (ii) may be useful for the manufacture of Product, including QA/QC testing and processes; and (iii) is proprietary to, or is maintained in confidence by, Novasep; and (b) patent and other intellectual property rights Controlled by Novasep that claim, cover or are appurtenant to any of the foregoing. Notwithstanding the foregoing or any other provision of this Agreement to the contrary, the parties hereby agree that "Novasep Background IP" specifically excludes: (x) all Confidential Information of Celladon disclosed to Novasep prior to the Effective Date, whether pursuant to the Confidentiality Agreement, pursuant to the Letter Agreement or otherwise; (y) all Celladon Materials; and (z) all Celladon Initial Project IP.

1.52 "Novasep Development Site" shall mean the Novasep facility owned and operated by Henogen and located at 12 rue des Professeurs Jeener et Brachet, B-6041, Gosselies, Belgium, which will be used to conduct some of the technology transfer and development work contemplated under this Agreement.

1.53 "Novasep Facility" shall mean the applicable manufacturing suite at Novasep's manufacturing facility owned and operated by Henogen and located at Rue de la Marlette n°14 (Zoning C), B-7180 Seneffe, Belgium, or any other manufacturing facility that is owned or controlled by Novasep or its Affiliate and is agreed to by Celladon in writing to be used to manufacture Product.

1.54 "Novasep Facility Modifications" shall mean the modifications to be made by Novasep and/or its subcontractors to the Novasep Facility, as described in Module 1 of the Scope of Work, including the procurement, installation and validation at the Novasep Facility of the equipment specified in Module 1b of the Scope of Work.

1.55 "Novasep Facility Modifications Costs" shall have the meaning set forth in Section 3.2.

1.56 "Novasep Project IP" shall mean any and all: (a) Information, inventions, developments and discoveries (whether or not patentable) developed, conceived, invented, first reduced to practice, made or generated solely by or on behalf of Novasep, any of its Affiliates, or any of Novasep's or its Affiliates' respective subcontractors, in the course of performance of the Services on or after the Effective Date, that, in each case, do not constitute Celladon New Project IP; and (b) patent and other intellectual property rights in or to any of the foregoing. Notwithstanding the foregoing or any other provision of this Agreement to the contrary, the parties hereby agree that "Novasep Project IP" specifically excludes: (x) all Confidential Information of Celladon disclosed to Novasep on or after the Effective Date, including without limitation, Celladon Background IP; (y) all Celladon Materials; and (z) all Celladon Project IP. For the sake of clarity, even if an item described in clause (a) of the first sentence of this Section 1.56 is developed or generated by or on behalf of Novasep […***…], such item will nonetheless be considered […***…], *provided* that (i) […***…] and (ii) […***…].

1.57 "Patented Novasep Background IP" shall have the meaning set forth in Section 8.3(b)(ii).

1.58 "PGT" shall have the meaning set forth in Section 2.1.

1.59 "POT" shall have the meaning set forth in Section 2.1.

1.60 "Product" shall mean bulk drug substance of Celladon's proprietary AAV1-based vector containing the expression cassette for SERCA2a, the active pharmaceutical ingredient of Mydicar.

1.61 "Product Warranty" shall have the meaning set forth in Section 9.3(b).

1.62 "Project Plan" shall have the meaning set forth in Section 2.2(b).

1.63 "Purchase Order" shall mean a purchase order submitted by Celladon in accordance with Section 4.3 for one or more cGMP Batches.

1.64 "Quality Agreement" shall mean the quality agreement to be entered into by the parties as of the Effective Date setting forth the respective responsibilities of the parties in relation to quality as required for compliance with cGMP, as the same may be amended from time to time by mutual written agreement of the parties. The parties shall cooperate in good faith and use Commercially Reasonable Efforts to complete and enter into the Quality Agreement by end of June 2015. To the extent the parties are unable to agree as provided above after using good faith efforts to do so for a period of sixty (60) days, such issue shall be referred to the Chief Executive Officers of the parties in accordance with Section 13.1, and thereafter, if not agreed, subject to Section 13.2. In the case of any conflict between this Agreement and the Quality Agreement, the terms of this Agreement shall control.

1.65 "Quality Control Procedures" shall mean the quality control and quality assurance program established by Novasep for Product manufactured hereunder, which shall be consistent with the Specifications and shall comply with Novasep's obligations under the Quality Agreement, applicable industry standards, cGMP and Applicable Law.

1.66 "Receiving Party" shall have the meaning set forth in Section 1.24.

1.67 "Regulatory Approval" shall mean any approvals (including supplements, amendments, pre-marketing and post-marketing approvals, and pricing and reimbursement approvals), licenses, registrations or authorizations of any Regulatory Authority or other governmental entity, necessary for the manufacture, distribution, use or sale of Product or Final Product in a regulatory jurisdiction.

1.68 "Regulatory Authority" shall mean the FDA in the United States, the EMA in the European Union or the applicable national regulatory authority in a European Union member state, as applicable, or the equivalent regulatory authority or entity having the responsibility, jurisdiction, and authority to approve the manufacture, use, importation, packaging, labeling, marketing and sale of pharmaceutical products in any other country or regulatory jurisdiction.

1.69 "Release" shall mean with respect to a Batch of Product the issuance by Novasep of a certificate of analysis and a certificate of compliance (cGMP Statement) for such Batch and the disposition of such Batch to release status.

1.70 "Scope of Work" shall mean the scope of work document attached to this Agreement as **Exhibit B**, which sets forth the parties' agreed plans and schedule for performing the technology transfer, equipment and materials purchase, equipment installation and validation, process development, scale-up and validation services, engineering and validation runs and other related activities as contemplated by this Agreement for preparing the Novasep Facility to be qualified for commercial manufacture of Product, as such scope of work may be amended from time to time by the PGT or by mutual written agreement of the parties.

1.71 "Second Extension Option" shall have the meaning set forth in Section 11.1(b).

1.72 "Specifications" shall mean the product attributes, characteristics, tests performed, acceptance criteria, storage and packaging requirements for Product consistent with the regulatory specifications described in Celladon's regulatory submissions for Product, to be mutually agreed by the parties before commencement of the production run for the first Engineering Batch and to be attached hereto as **Exhibit C** (which will be incorporated herein by this reference), as the same may be amended or supplemented from time to time in accordance with this Agreement. To the extent the parties are unable to agree as provided above after using good faith efforts to do so for a period of […***…] ([…***…]) days, such issue shall be referred to the Chief Executive Officers of the parties in accordance with Section 13.1, and thereafter, if not agreed, subject to Section 13.2.

1.73 "Supply Failure" shall mean the failure by Novasep to supply the […***…], conforming to the Product Warranty, ordered by Celladon […***…] by, or within […***…] days after, the delivery date in the applicable Manufacturing Schedule, except to the extent such failure is due to […***…] or to […***…] or the […***…].

1.74 "**Take or Pay Compensation**" shall have the meaning set forth in Section 4.4(c).

1.75 "Technology Transfer Plan" shall mean the written plan for the transfer by Celladon to Novasep of the technology, processes and analytics necessary to enable Novasep to produce Product on behalf of Celladon, including the schedule for completion of such transfer, the initial version of which plan is set forth in the Scope of Work, and which may be amended from time to time by the POT or the PGT in accordance with Article 2.

1.76 "Term" shall have the meaning set forth in Section 11.1.

1.77 "Third Party" shall mean any entity or individual other than Celladon and Novasep and their respective Affiliates.

1.78 "Validation Batch" shall mean a Batch that is produced with the intent to show reproducibility of the Manufacturing Process and is required to complete process validation studies.

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1.79 "Validation Criteria" shall have the meaning set forth in Section 3.5.

2. PROJECT MANAGEMENT AND GOVERNANCE

2.1 Objective. Celladon and Novasep acknowledge and agree that effective communication between the parties at both the operational level and the management level is essential for the achievement of the objectives of the Project. In order to facilitate such communication, the parties shall establish a Project Operational Team (***"POT"***) and a Project Governance Team (***"PGT"***), having the respective responsibilities set forth below in this Article 2. Each party shall be responsible for ensuring that, at all times, its representatives on the POT and PGT act reasonably and in good faith in carrying out their respective responsibilities hereunder.

2.2 Project Operational Team. The POT shall be composed of appropriate Celladon and Novasep technical experts having operational responsibility within their respective organizations for the implementation and performance of the Scope of Work. The POT shall meet, in person or by teleconference or videoconference, at regular intervals specified in the Scope of Work (or at such other intervals as the POT deems necessary and appropriate). The POT will operate by consensus with each party's POT representatives collectively having one vote. All meetings of the POT shall be documented in written minutes generated initially by a representative of Novasep, and later, upon request of Celladon, by each party, on an alternating basis, beginning with Celladon, and in any case circulated within two Business Days of the POT's meeting, and then approved by both parties' representatives on the POT. The POT's overall responsibility shall be to encourage and facilitate ongoing cooperation and communication between the parties regarding the progress and results of Scope of Work activities. Other responsibilities of the POT shall include:

(a) periodically reviewing and, from time to time as necessary or appropriate, preparing and recommending to the PGT for approval amendments and updates to the Scope of Work;

(b) as the POT deems necessary or appropriate, developing and approving, and thereafter overseeing the completion of from time to time, one or more individual project plans for specific Scope of Work activities setting forth the technical details of such activities, including, without limitation, technical specifications of deliverables from such activities, and the schedule for performance of such activities or delivery of such deliverables (each, a ***"Project Plan"***), including, without limitation, the Technology Transfer Plan, provided that each such Project Plan and any amendment thereto shall be consistent with the then-current Scope of Work; and

(c) discussing and attempting in good faith to resolve technical issues that may arise in the performance of the Scope of Work or any Project Plan, including the Technology Transfer Plan, and attempting to resolve any disagreements as to matters that are contemplated under the Scope of Work to be agreed upon by the parties.

If the POT is unable to reach consensus regarding any matter for which it is responsible, such matter shall be referred to the PGT for attempted resolution. Within one Business Day after

each POT meeting, Novasep shall deliver to the POT and the PGT a written progress report regarding the Development Services, as more fully described in the Scope of Work.

2.3 Project Governance Team. The PGT shall be composed of an equal number of appropriate members of Celladon's and Novasep's respective management teams having appropriate technical credentials, experience, knowledge, and decision-making authority within their respective organizations. The PGT shall meet, in person or by teleconference or videoconference, at regular intervals specified in the Scope of Work and on an *ad hoc* basis as necessary from time to time. Each party shall designate one of its PGT representatives to serve as a co-chair of the PGT, and to that end, Paul Cleveland shall be the co-chair from Celladon, and Jerome Bedier will be the co-chair from Novasep as of the Effective Date. The parties' respective PGT co-chairs or their designees shall be responsible for jointly preparing meeting materials for each regularly scheduled PGT meeting, including an agenda setting forth the topics and issues to be addressed at such meeting and updates regarding the status, progress and results of Project activities and sending such materials to all PGT members five business days before the applicable meeting, and all meetings of the PGT shall be documented in written minutes generated by a party's co-chair, on an alternating basis, beginning with Novasep, and circulated within two Business Days of the PGT's meeting, and then approved by both parties' representatives on the PGT. Subject to Section 2.4, the PGT shall have decision-making authority with respect to major matters relating to the Project, including, without limitation:

(a) approval of amendments to the Scope of Work;

(b) prioritization of efforts, including work sequences, under the Scope of Work;

(c) discussion and approval of corrective actions to address breakdowns or deficiencies in communications between the parties relating to the Project or other significant issues arising in the performance of the Project; and

(d) resolving disputes referred to it by the POT, including any disputes arising between the parties with respect to matters to be agreed upon by the parties under the Scope of Work.

Decisions of the PGT shall be made by unanimous vote with each party's PGT representatives collectively having one vote. No vote of the PGT may be taken unless at least one of each party's representatives is present for the PGT vote. If, despite the good faith efforts of the parties' representatives on the PGT, the PGT is unable to reach a unanimous decision with regard to any matter within its authority within a reasonable period, then such matter shall be referred to the Chief Executive Officer of Celladon and the Head of the "Biopharma Business Unit" of Novasep, who shall promptly meet and attempt in good faith to resolve such matter within fifteen (15) days. If such officers are unable to resolve such matter within such fifteen-day period, the matter shall be resolved by binding arbitration in accordance with Section 13.2. Within three (3) Business Days after each PGT meeting, Novasep shall draft and provide to Celladon for review a report of such PGT meeting, which Celladon shall review and approve or modify (as applicable) within two Business Days of receipt and which shall be finalized and released no later than five Business Days after such PGT meeting.

2.4 Limitations on POT and PGT Authority. Neither the POT nor the PGT shall have any right, power or authority:

(a) to determine any issue in a manner that would conflict with the express terms and conditions of this Agreement;

(b) to modify or amend the terms and conditions, or waive any provision, of this Agreement; or

(c) to amend or modify the amount or timing of any payment obligation of Celladon to Novasep hereunder or to impose any additional payment obligation on Celladon.

3. DEVELOPMENT SERVICES

3.1 Technology Transfer. Under the terms of the Letter Agreement, the parties initiated a program of transferring specific technology and materials relating to the manufacture of Product to enable the commencement of manufacturing scale-up and validation by Novasep promptly after the entry into this Agreement. The parties shall use Commercially Reasonable Efforts to complete, as promptly as practicable after the Effective Date, the transfer to Novasep of such Celladon Background IP, including Celladon Materials, as are required for commencement of such manufacturing scale-up and validation by Novasep, and such existing development reports, previous campaign reports, previous transfer reports and other data and documentation necessary to support the validation strategy set forth in the Scope of Work, in each case, in accordance with the Technology Transfer Plan. Novasep shall keep Celladon regularly and fully informed of Novasep's progress in completing the technology transfer, including permitting appropriate Celladon employees or representatives to visit and inspect the Novasep Development Site or the Novasep Facility in connection with conducting such work. Subject to Celladon's payment obligations set forth in Section 7.1, each party shall bear its own costs in effecting and completing such technology transfer.

3.2 Facility Modification; Equipment Purchase and Installation. Novasep undertakes and agrees to invest in, and to complete as soon as reasonably possible, the modifications to the Novasep Facility described in Module 1 of the Scope of Work, including the procurement, installation and validation at the Novasep Facility of the equipment specified in Module 1b of the Scope of Work. It is foreseen that the overall costs for such modification by Novasep and/or its subcontractors of the Novasep Facility Modifications will not be greater than EUR […***…] ([…***…]€) (the "**Novasep Facility Modifications Costs**"), which amount shall be paid by Celladon to Novasep during the period in which Novasep delivers the Development Services, and as part of the Development Services fees paid pursuant to Section 7.1(a), including EUR […***…] ([…***…] €) of the Novasep Facility Modifications Costs, which are […***…]. Novasep will use Commercially Reasonable Efforts (i) to complete such facility modifications, procurement, installation and validation on a schedule that permits completion of validation of the Manufacturing Process and initiation of cGMP manufacture of Product at the Modified Novasep Facility substantially in accordance with the schedule set forth in the Scope of Work, and (ii) to maintain all costs and expenses relating to such facility modifications, procurement, installation and validation below the Novasep Facility

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Modifications Costs. Celladon shall use Commercially Reasonable Efforts to cooperate with Novasep in its efforts to complete such activities. Novasep shall provide documentation to support the engineering design of the modifications to the Novasep Facility, and all Novasep Facility Modifications Costs, and Celladon shall review and sign off on the required documents relating to such facility modifications and equipment validation as specified in the Quality Agreement or as otherwise agreed to by the parties in writing prior to the execution of the Quality Agreement and shall use Commercially Reasonable Efforts to complete such sign off within […***…] ([…***…]) Business Days of receipt of the documents and all information needed for such review and sign off, provided that Novasep has submitted to Celladon drafts of such documents as soon as reasonably available. It is understood that such Novasep Facility Modifications and equipment procurement, installation and validation are essential to enable Novasep to commercially manufacture Product at the Novasep Facility on behalf of Celladon in the manner and within the timelines specified in the Scope of Work and this Agreement. Subject to Celladon's payment obligations set forth in Section 7.1, Novasep shall pay all Novasep Facility Modifications Costs, and all Novasep Facility Modifications shall be solely and fully owned by Novasep.

3.3 Use of the Modified Novasep Facility; Celladon's Right of First Refusal.

(a) The Modified Novasep Facility will be designed to be capable of production of multiple products, including the Product; provided, however, that Celladon shall have until […***…] to determine, by written notice to Novasep, in its sole discretion whether to permit the Modified Novasep Facility to be multi-purpose, or whether to have the facility be maintained as a dedicated facility.

(b) If Celladon so elects the Modified Novasep Facility to be multi-purpose, then Novasep will have the right to use the Modified Novasep Facility not only for the manufacture of Products for Celladon, but also for the manufacture of other products (for itself or any of its other customers), provided that: (i) the use of the Modified Novasep Facility during the Term for a product other than Product does not impact Novasep's ability to deliver to Celladon Product conforming to the Specifications and manufactured and released in compliance with cGMP in the quantities and on the schedule specified in Celladon's then-pending Purchase Orders and the Binding Period of the current Forecast; (ii) prior to Regulatory Approval of Product, any use of the Modified Novasep Facility during the Term for any product other than Product does not delay, or impair Celladon's ability to obtain, Regulatory Approval of Product; (iii) prior approval has been obtained from all relevant Regulatory Authorities for the manufacture of Product in a multi-product facility; and (iv) Novasep can demonstrate to Celladon's reasonable satisfaction that it is able to effectively clean the production facility so as to avoid cross contamination of the Product with other products. In addition, Novasep and Celladon shall agree in writing on changeover and cleaning procedures, testing, and necessary qualification to support multi-product use of the Modified Novasep Facility.

(c) If Celladon elects instead under Section 3.3(a) that the Novasep Modified Facility be a dedicated facility (i.e. the Novasep facility, including the Novasep Facility Modifications), then the following provisions shall apply:

(i) The First Extension Option shall be deemed as granted by Celladon; and

(ii) Celladon shall pay to Novasep each calendar year during the Term an amount (the **"Make-Whole Amount"**) corresponding to […***…] percent ([…***…]%) of the applicable Batch Price of the Batches of Product not produced in the unused capacity of the dedicated Novasep Facility. The unused capacity of the dedicated Novasep Facility corresponds to the difference between the Maximum Capacity (which for purposes of this calculation means […***…] Batches / calendar year unless mutually agreed upon by the parties) less the number of Batches ordered by Celladon for such calendar year pursuant to a Purchase Order under Section 4.4, or the minimum number of Batches to be purchased by Celladon in that given calendar year pursuant to Section 4.4, whichever is the greater. Notwithstanding the foregoing, such Make-Whole Amount shall not be due and owing for any calendar year, or portion thereof, during which there exists any shut-down or stoppage of the Modified Novasep Facility, for whatever reason, other than stoppage for planned preventive maintenance for up to […***…] days per calendar year as contemplated by Section 1.47.

(d) Notwithstanding Novasep's potential rights under Section 3.3(b), during the Term: (i) Novasep shall not manufacture any other product (for itself or any other customer) within the Modified Novasep Facility concurrently with the manufacture of Product; (ii) Novasep shall use the Modified Novasep Facility in priority for the purpose of conducting the Development Services on behalf of Celladon and performing Novasep's manufacturing and supply obligations with respect to quantities of Product set forth in Celladon's then-pending Purchase Orders and binding Forecasts; and (iii) at all times during each calendar year of the then-current Term, Novasep shall reserve sufficient capacity in the Modified Novasep Facility to manufacture the number of Batches that Celladon is required to purchase and pay for (or, as applicable, pay the Take or Pay Compensation for) under Section 4.4.

(e) Subject to Novasep's compliance with Sections 3.3(b) and 3.3(d), should Novasep have any available unused capacity in the Modified Novasep Facility during the Term, then if Novasep's rights under Section 3.3(b) become effective, before making that capacity available for use for other products (of Novasep or any of its other customers), Novasep shall first provide written notice to Celladon setting forth the amount of available capacity, the period for which it is available and the number of Batches of Product required to fill such available capacity, and offering to make that capacity available to Celladon for the manufacture of the Product. In the event that Celladon wishes to have Novasep use the specified capacities (or a portion thereof) for the manufacture of the Product, it shall provide notice in writing of such election within […***…] ([…***…]) business days of its receipt of Novasep's notice of available capacity, which Celladon election notice shall specify the number of Batches (not to exceed the number set forth in Novasep's notice of available capacity) and be accompanied by a Purchase Order for such number of Batches, and in such case Novasep will use such available capacity in the Modified Novasep Facility for the manufacture of Product in accordance with such Purchase Orders and the terms of this Agreement. If Celladon does not timely elect to have Novasep use the specified capacities (or any portion thereof) for the manufacture of the Product as set forth above, then, subject to Novasep's compliance with Sections 3.3(a) and 3.3(b), Novasep will have the right to use such capacities (or the portion thereof that is not needed to manufacture the number of Batches in Celladon's election notice, as applicable) in the Modified Novasep Facility

for other projects and products (of Novasep or any of its other customers) and, notwithstanding the provisions of Section 4.3, Novasep will have no obligation to accept any Purchase Order from Celladon which would require the use of any such capacity.

If Novasep's use of the Modified Novasep Facility for other products (of Novasep or any of its other customers) results in Novasep not having sufficient capacity in the Modified Novasep Facility to manufacture the applicable minimum number of Batches that Celladon is required to purchase and pay for (or, as applicable, pay the Take or Pay Compensation for) under Section 4.4 for any calendar year of the Term, such failure to preserve sufficient capacity shall be considered a material breach of this Agreement by Novasep, entitling Celladon, at its sole option, to terminate this Agreement pursuant to Section 11.3(a), and if Celladon elects not to exercise such right to terminate this Agreement, Celladon shall be relieved of its obligations under Section 4.4 to purchase a minimum number of Batches, solely with respect to the number of Batches that Novasep was unable to manufacture as a result of such use of the Modified Novasep Facility for other products.

3.4 Process Development and Scale-Up; Engineering Batches. Commencing promptly after completion of the Technology Transfer Plan, Novasep shall:

(a) perform, at (i) the Novasep Development Site or (ii) subject to completion of the Novasep Facility Modifications and equipment procurement, installation and validation activities contemplated by Section 3.2, the Modified Novasep Facility (as applicable, as specified in the Scope of Work), the process development and scale-up activities for Product described in Modules 3 to 6 of the Scope of Work and in any Project Plan(s) for such activities or study(ies) that may be approved by the POT; and

(b) manufacture Engineering Batches at the Novasep Facility until it has completed […***…] ([…***…]) Engineering Batches that conform to the Engineering Batch Specifications, unless the parties mutually agree that a lesser number of successful Engineering Batches has sufficiently demonstrated that the Manufacturing Process has been successfully transferred to the Modified Novasep Facility and could be operated therein in compliance with cGMP. The parties will mutually agree to the Engineering Batch Specifications prior to commencement of production of the first Engineering Batch and after Novasep completes process transfer and has process experience from process studies. The parties acknowledge […***…].

Novasep shall use Commercially Reasonable Efforts to complete such process development and scale-up activities and such study(ies) and to manufacture the required number of Engineering Batches on a schedule that permits the initiation of validation studies, the manufacture of Validation Batches and the validation of the Manufacturing Process at the Novasep Facility substantially in accordance with the schedule set forth in the Scope of Work. Novasep shall disclose to Celladon in written reports all results of such activities and studies and all related deliverables (including the Engineering Batches) as required by the Scope of Work and any applicable Project Plans.

3.5 Validation Batches. Prior to commencement of manufacture of Validation Batches as described below in this Section 3.5, the parties shall mutually agree in good faith on

appropriate success criteria for the Validation Batches, which shall be documented in a POT-approved Project Plan and shall include without limitation conformity to the Specifications (***"Validation Criteria"***). Promptly after POT approval of such Project Plan, Novasep shall manufacture in compliance with cGMP and deliver to Celladon such number of Validation Batches meeting the Validation Criteria as is mutually agreed by the parties to be sufficient to document the operability and reproducibility of the Manufacturing Process and to permit the parties to complete and file the regulatory documents necessary for commercial manufacture of cGMP-compliant Product, as described in the Scope of Work and any applicable Project Plans. The parties acknowledge that successful Validation Batches may be commercially sold by or on behalf of Celladon after validation has been achieved. If a particular Validation Batch supplied by Novasep fails to meet the Validation Criteria or to conform to the Specifications, appropriate representatives from each party shall meet and discuss and seek to determine the causes of such Validation Batch having failed to meet the Validation Criteria or conform to the Specifications and […***…]. The parties acknowledge and agree that […***…].

3.6 Pre-Approval Regulatory Support. Novasep shall perform and support any pre-approval regulatory activities (including preparing for and cooperating with any pre-approval inspection by FDA, EMA or any other applicable Regulatory Authority) reasonably requested by Celladon to support the filing by Celladon, any of its Affiliates or any Licensee of applications for, or to obtain, Regulatory Approval, for Product manufactured at the Novasep Facility, or Final Product containing Product manufactured at the Novasep Facility (as applicable), in the United States, the European Union or any of its member states, and any other country or regulatory jurisdiction mutually agreed by the parties. Celladon shall compensate Novasep for such pre-approval regulatory support activities as set forth in the Scope of Work or the applicable Project Plan, provided that any such regulatory support activities in connection with applications for Regulatory Approval or Regulatory Approvals outside of the United States and the European Union, including the costs or compensation payable by Celladon therefor, shall be subject to prior written agreement of the parties.

3.7 Stability Studies. Celladon shall conduct stability studies on Product from the Engineering Batches manufactured by Novasep hereunder in accordance with study protocols approved by the POT prior to Novasep commencing the manufacture of Validation Batches under Section 3.5 above. Celladon shall prepare and deliver to Novasep written reports setting forth the results of such stability studies.

3.8 Language of Reports and Documents. Novasep shall provide to Celladon the following documents and reports in English or in bilingual version (English and French), at no additional cost beyond the payments specified in Section 7.1.:

[…***…]

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[…***…]

For Novasep's […***…].

[…***…].

In addition, if requested by Celladon, Novasep will provide translation into any language other than English of any documents relating to manufacture of Product, for regulatory or any other purposes, and the reasonable costs for any such additional translations shall be borne by Celladon.

3.9 Additional Services. Any services not contemplated by the Scope of Work, including the compensation to be paid by Celladon to Novasep for such services, would be separately negotiated in good faith and mutually agreed by the parties in writing.

4. COMMERCIAL MANUFACTURE AND SUPPLY OF PRODUCT

4.1 Manufacture and Supply; Language of cGMP Documents. Except as set forth in Section 3.4 in the case of Engineering Batches, Novasep shall manufacture at the Novasep Facility and supply to Celladon cGMP Batches of Product conforming to the Product Warranty in such quantities as ordered by Celladon in Purchase Orders submitted to Novasep by Celladon in accordance with Section 4.3, subject to the minimum annual purchase commitments set forth in Section 4.4. Novasep shall manufacture all such Product in compliance with cGMP and in accordance with the Manufacturing Process and shall complete and deliver to Celladon the Batch Documentation for each Batch. Novasep's manufacturing of a particular Batch shall be deemed completed at such time as Novasep completes those release activities for such Batch for which Novasep is responsible under the Quality Agreement. For clarity, Celladon shall be solely responsible for the manufacture and release of Final Product incorporating the Product supplied hereunder, including all fill/finish activities and all packaging and labeling of Final Product.

4.2 Language of GMP Documents. Novasep shall provide to Celladon the following documents and reports with respect to cGMP Batches in English or in bilingual version (English and French), at no additional cost beyond the payments specified in Section 7.1:

[…***…]

[…***…]

For Novasep's […***…].

[…***…].

In addition, if requested by Celladon, Novasep will provide translation into any language other than English of any documents relating to manufacture of Product, for regulatory or any other purposes, and the reasonable costs for any such additional translations shall be borne by Celladon.

4.3 Purchase Orders. To order Batches of Product to be supplied by Novasep, Celladon shall provide to Novasep written Purchase Orders specifying the number of Batches of Product ordered, the delivery destination(s) and the requested delivery dates for the particular Batches. The quantity of Product ordered in each Purchase Order shall be in whole numbers of Batches. No later than […***…] days after Celladon's delivery to Novasep of the Initial Forecast, Celladon shall submit a written Purchase Order ordering the Batches of Product forecasted for the Binding Period of the Initial Forecast. On a quarterly basis thereafter during the Term, no later than seven days after delivery of each Forecast pursuant to Section 4.5, Celladon shall submit a written Purchase Order for the number of Batches of Product forecasted for the last quarter of the Binding Period of such Forecast. Promptly after the submission of the Purchase Order for the Binding Period of the Initial Forecast and on or about […***…] of each year thereafter during the Term, the parties shall meet and discuss in good faith and agree reasonably on the timing and schedule of the manufacturing campaigns to be conducted by Novasep during the following calendar year to manufacture and deliver the total number of Batches ordered for such calendar year and on the delivery schedule for each of the Batches resulting from such manufacturing campaign (such agreed schedule for a particular calendar year, the ***"Manufacturing Schedule"***). Each Manufacturing Schedule shall include the expected delivery dates by calendar quarters of the Batches ordered for the calendar year. Novasep shall accept and shall be deemed to accept each Purchase Order submitted by Celladon in accordance with the terms of this Agreement, subject to the Maximum Capacity. Purchase Orders for Product submitted by Celladon shall reference this Agreement and shall be governed exclusively by the terms contained herein. Any provision in any Purchase Order, invoice, or similar document furnished by Celladon or Novasep that is in any way inconsistent with the terms and conditions set forth in this Agreement is hereby rejected, unless otherwise expressly agreed by the parties in writing.

4.4 Minimum Purchase Commitments.

(a) Initial Term. Subject to the terms and conditions of this Agreement, Celladon hereby agrees to purchase and pay for, and to submit Purchase Orders for, at least the minimum number of Batches of Product as set forth below during 2017 and 2018 at the applicable Batch Price specified in Section 7.2, subject to Section 7.3:

Calendar Year	Minimum Number of Batches
2017	[***]
2018	[***]

(b) Extension Terms. Subject to the terms and conditions of this Agreement, if Celladon exercises its option to extend the Term by one or both Extension Terms, Celladon hereby agrees to purchase and pay for, and to submit Purchase Orders comprising Annual Orders for the purchase of, at least the minimum number of Batches of Product as set forth below during (i) 2019 if Celladon exercises the First Extension Option and (ii) 2020 if Celladon exercises the Second Extension Option, in each case, at the applicable Batch Price for such year specified in Section 7.2, subject to Section 7.3:

Calendar Year	Minimum Number of Batches
2019	[***]
2020	[***]

(c) Take-or-Pay. In the event that, in any calendar year, Celladon submits Purchase Orders for fewer Batches than the applicable minimum Batch commitment for such year set forth in Section 4.4(a) or 4.4(b), as applicable, Celladon shall be obligated to pay to Novasep compensation as follows: the difference between the actual quantity of Batches ordered by Celladon and the above mentioned minimum number of Batches will be invoiced by Novasep to Celladon at a price corresponding to […***…]% ([…***…] percent) of the then applicable Batch Price (the "***Take or Pay Compensation***"). Such invoice(s) relating to non-purchased quantities will be paid by Celladon to Novasep no later than January 15th of the following year.

As an example, if Celladon purchases only […***…] ([…***…]) Batches of Product in 2018, Celladon shall pay to Novasep a Take or Pay Compensation of […***…] ([…***…]) Batches x [[…***…] Euros] = […***…] EUR, the corresponding invoice to be paid to Novasep no later than January 15th 2019.

4.5 Forecasts. No later than […***…] 1, 2016, and the first day of each calendar quarter thereafter during the Term, Celladon shall provide Novasep with rolling […***…]-month (except as set forth below) forecasts setting forth its good faith estimate, by month, of its expected orders and Batch requirements for Product during the applicable period (each, a ***"Forecast"***) as of the date of such Forecast, with the first such Forecast to cover the […***…]-month period beginning on January 1, 2017 (the ***"Initial Forecast"***); *provided, however,* that:

(a) the forecasted number of Batches for any calendar year shall not exceed the then-applicable Maximum Capacity;

(b) the first […***…] months period of each Forecast shall be binding upon Celladon (each a "***Binding Period"***);

(c) a Forecast need not cover any period after expiration of the then-current Term; and

(d) if any Forecast provided by Celladon does cover any period after expiration of the then-current Term, the forecasted Product orders for such post-expiration period shall be non-binding.

4.6 Delivery Date. For each Purchase Order submitted by Celladon in accordance with the terms of this Agreement, Novasep shall deliver to Celladon or Celladon's designee, at the delivery destination and by the delivery date specified in such Purchase Order (or such other delivery date as agreed by the parties), the specified quantity of Product conforming to the Product Warranty and complying with the other applicable requirements under this Agreement. Novasep shall immediately report to Celladon the occurrence of any event within or beyond its control which is likely to affect delivery of any order of Product, provided that the giving of such notice shall not relieve Novasep of its obligations hereunder.

4.7 Shortfalls in Supply.

(a) If at any time following commencement of cGMP manufacture of Product at the Novasep Facility, Novasep believes that it will be unable to deliver the number of Batches ordered by Celladon for delivery during any period, Novasep shall promptly provide written notice thereof to Celladon, which notice shall include (i) the number of Batches that Novasep believes it will be unable to deliver, (ii) the reasons for Novasep's inability to deliver such number of Batches and (iii) Novasep's anticipated timeline for being able to deliver such number of Batches (such notice, a ***"Shortfall Notice"***). Following delivery of a Shortfall Notice, Novasep shall be obligated to provide written notice(s) to Celladon promptly in the event there are subsequent changes in the details covered by a particular Shortfall Notice (*e.g.*, if Novasep subsequently learns that it will be able to deliver a greater or lesser number of Batches than previously described in the Shortfall Notice or any prior update notice, or if Novasep's anticipated timelines for curing such shortfall change).

(b) Upon the occurrence of any Supply Failure, the parties, via the POT or PGT, shall meet immediately and discuss in good faith all appropriate actions to remedy and cure the Supply Failure. In any event Novasep shall use best efforts to cure the Supply Failure as soon as possible (and in any event within […***…] ([…***…]) months), shall not manufacture any product other than Product in the Novasep Facility until such Supply Failure is cured and shall provide Celladon with regular and accurate reports on the status of its efforts and the actual date that it expects to deliver the required amounts of Product on order.

(c) If a Supply Failure occurs, (i) Celladon shall be […***…]; and (ii) Novasep's rights under […***…], in each case until such time as such Supply Failure is cured.

4.8 Labeling and Packaging. Novasep shall label and package Product supplied hereunder in accordance with the applicable Manufacturing SOPs, the Quality Agreement, Celladon's reasonable directions and Applicable Law regarding pharmaceutical products shipped in bulk.

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4.9 Storage and Handling. Novasep shall store and handle all Product as required by the applicable Manufacturing SOPs, cGMP, and all established safety practices for the Product.

4.10 Shipping; Risk of Loss. All shipments of Product by Novasep will be made FCA (Incoterms 2010) the Novasep Facility (regardless of whether or not Novasep provides assistance to Celladon for the organization of the shipment of Product and/or chooses the shipping agent and common carrier on behalf of Celladon) to the delivery destination specified in the applicable Purchase Order, and all deliveries shall be in accordance with the shipping instructions of Celladon and, unless otherwise agreed by the parties, using a common carrier specified by Celladon. Novasep shall pay, on Celladon's behalf, all costs of shipping, storage, customs, duties, taxes, freight, insurance and other charges incurred by Novasep in shipping Product (collectively, ***"Transport Costs"***), unless Celladon provides otherwise in writing on a Purchase Order. Celladon will reimburse Novasep for the reasonable Transport Costs, without mark-up, actually incurred by Novasep, and Novasep shall, upon Celladon's request, provide written documentation supporting such costs. Except as provided herein with respect to any Non-Conforming Batch, risk of loss as to Product shipped to Celladon or its designee hereunder shall pass to Celladon upon delivery of such Product to the common carrier at the Novasep Facility where the Product was manufactured. Novasep shall arrange to insure the shipment of the ordered Product as specified by Celladon, in the name and on behalf of Celladon, at Celladon's costs. Title to Product delivered hereunder shall pass to Celladon upon payment to Novasep of the corresponding invoice.

4.11 Celladon Materials. Celladon shall be responsible for ensuring that adequate quantities of Celladon Materials, of appropriate quality, meeting the Celladon Materials Specifications, are delivered to the Novasep Facility free of charge in sufficient time for Novasep's manufacture of the quantities of Product ordered by Celladon. Novasep shall not be liable for any failure to perform or delay in performing its obligations hereunder to the extent the late arrival of Celladon Materials, or the quality of Celladon Materials, adversely affects Novasep's ability to perform such obligations. Celladon shall promptly provide written notice to Novasep of the occurrence of any event within or beyond Celladon's control which is likely to prevent Celladon from complying with its obligations under the first sentence of this Section 4.11, provided that the giving of such notice shall not relieve Celladon of its obligations hereunder.

4.12 Raw Materials. Novasep shall be responsible for procuring all raw materials needed for manufacturing Product, except for the Celladon Materials. The parties shall jointly develop and agree on applicable specifications for raw materials. Novasep shall source raw materials and consumables solely from qualified vendors as specified in the Quality Agreement, and shall provide Celladon with the names of all such vendors. If Celladon specifies a particular vendor for any such raw material, Novasep shall procure such raw material solely from such Celladon-specified vendor. Novasep shall conduct audits of vendors as required by the Quality Agreement or applicable regulatory requirements or as reasonably requested by Celladon.

4.13 Staffing and Resources. Novasep shall establish the necessary organization, and shall at all times during the Term use Commercial Reasonable Efforts to allocate sufficient time, effort and resources, and use personnel with sufficient skills and experience, as, in each case, are

required to perform Novasep's Product manufacturing and supply obligations under this Article 4 on a timely basis and in accordance with the terms of this Agreement.

4.14 Third Party Suppliers. Novasep acknowledges that, prior to the Collaboration Initiation Date, Celladon entered into a facility construction and commercial supply agreement for Product with a Third Party and that, under such agreement, Celladon may become obligated to order from such Third Party a certain percentage of Celladon's and its Licensee's annual global supply of Product (subject to certain limits and adjustments set forth in such agreement). Novasep further acknowledges and agrees that Celladon may enter into one or more additional commercial manufacturing and supply agreements with Third Parties for Product, without restriction, subject only to Celladon's compliance with its obligations under Section 4.4. Accordingly, the parties agree that Celladon shall have no obligation to purchase any particular percentage of its commercial requirements of Product from Novasep hereunder, subject only to Celladon's compliance with its obligations under Section 4.4.

5. QUALITY ASSURANCE AND ACCEPTANCE

5.1 Quality. Immediately upon manufacture of a Batch, Novasep shall conduct those analytical tests of such Batch for which Novasep is responsible as described in the Specifications, and shall deliver to Celladon or its designee sample(s) of such Batch as required by the Specifications (the ***"Batch Sample"***). Celladon will provide the analytical test results of its testing of the Batch Sample to Novasep for inclusion in the Batch Documentation. Prior to the delivery of each Batch of cGMP Product, Novasep shall deliver to Celladon the Batch Record and Master Batch Record for such Batch and such other documentation with respect to such Batch as specified in the Quality Agreement (the ***"Batch Documentation"***).

5.2 Acceptance and Rejection.

(a) Promptly after Celladon's or its designee's receipt of all associated Batch Documentation for a Batch, Celladon shall review such Batch Documentation to determine whether or not it conforms to the Product Warranty. Celladon shall notify Novasep in writing of Celladon's determination as to the conformity or non-conformity of the Batch to the Product Warranty, in each case by the later of (i) […***…] days after receipt of the Batch Sample at Celladon's designated testing site and (ii) […***…] days after Celladon's or its designee's receipt of the Batch Documentation, or such other period as may be specified in the Quality Agreement (as applicable, the ***"Acceptance Period"***). The Acceptance Period shall be subject to extension by mutual written agreement of the parties for a reasonable period in the event of delays in receipt of test results or if additional testing or investigations into deviations from cGMP are necessary. If the results of such analytical testing demonstrate that the Batch Sample fails to conform to Specifications, or it is determined that manufacturing of Product failed to meet the Product Warranty, Celladon shall notify Novasep in writing thereof before expiration of the applicable Acceptance Period (as extended, if applicable), after which time, if Celladon has not delivered written notice of rejection, such Batch shall be deemed accepted by Celladon.

(b) If Novasep believes that a Batch has been improperly rejected, appropriate quality representatives of the parties shall promptly discuss the matter in good faith and attempt to reach mutual agreement as to the conformity or non-conformity of the Batch Sample to the

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Specifications, or if cGMP deviations are material and may reasonably be expected to impact product or process, or otherwise fail the Product Warranty and as to the cause(s) of any non-conformity. If such representatives are unable to reach mutual agreement within […***…] days, the parties shall, as applicable, cause an independent laboratory reasonably acceptable to both parties, to perform testing of samples of such Batch to determine whether or not such Batch conforms to the Specifications and/or cause an independent GMP expert reasonably acceptable to both parties to review Batch Documentation for such Batch to determine whether or not such Batch was manufactured in accordance with cGMP and, if ascertainable, to determine the cause(s) of such non-conformity. The independent laboratory's and/or independent GMP expert's determination shall be final and binding on the parties. The parties shall initially share the costs associated with such testing and review equally, but the party against whom the independent laboratory and/or independent GMP expert rules shall reimburse the other party for the share of such costs initially borne by the other party within 30 days after the independent laboratory and/or independent GMP expert issues its determination.

(c) Celladon shall not be required to pay for cGMP Batch that does not conform to the Product Warranty (a ***"Non-Conforming Batch"***), provided that if Celladon previously paid for such Non-Conforming Batch, then Novasep shall, at Celladon's option, either (i) replace the Non-Conforming Batch with a cGMP Batch that conforms to the Product Warranty in accordance with Section 5.2(d), or (ii) if Celladon elects not to have Novasep provide a replacement cGMP Batch, issue a credit to Celladon in the amount of such payment or refund to Celladon the amount of such payment, at Celladon's election, within 30 days of Celladon's written request therefor.

(d) If Celladon elects to have Novasep replace a Non-Conforming Batch with a cGMP Batch that conforms to the Product Warranty, then Novasep shall replace such Non-Conforming Batch as promptly as practicable, and the parties' respective rights and obligations with respect to such Non-Conforming Batch shall be as set forth below.

(i) If the failure of a Non-Conforming Batch to conform to the Product Warranty (A) is primarily attributable to Novasep's breach of its obligations hereunder or Novasep's gross negligence or intentional misconduct or (B) results from a critical failure at the Novasep Facility or in the performance of the Manufacturing Process or the equipment used to manufacture Product that, in each case, is primarily attributable to Novasep's breach of its obligations hereunder or Novasep's gross negligence or intentional misconduct, then Novasep […***…].

(ii) Except as expressly set forth in Section 5.2(d)(iii), if the failure of a Non-Conforming Batch to conform to the Product Warranty is attributable to any cause other than those specified in Section 5.2(d)(i), Novasep shall […***…]

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[…***…].

(iii) If the failure of a Non-Conforming Batch to conform to the Product Warranty is primarily attributable to the failure of the Celladon Materials provided by Celladon for use in the manufacture of such Batch to conform to the Celladon Materials Warranty at the time of delivery to Novasep, then, provided that Novasep performed the applicable testing of such Celladon Materials in accordance with the Quality Agreement prior to using such Celladon Materials in the manufacture of such Batch, Novasep shall have no liability or responsibility for such Non-Conforming Batch under the preceding provisions of this Section 5.2 and Celladon shall […***…].

(e) The parties shall mutually agree in good faith on how (if at all) any Non-Conforming Batch may be used and, if the parties agree that such Non-Conforming Batch is not usable, on the disposition of such Non-Conforming Batch, including the costs thereof.

(f) Celladon acknowledges and agrees that, except for Celladon's rights to terminate this Agreement under Article 11, Celladon's sole remedy with respect to a Non-Conforming Batch is as set forth above in this Section 5.2.

6. REGULATORY

6.1 Facilities Licenses. Novasep shall obtain and maintain, at its sole cost, all permits, licenses and approvals (including facilities licenses) necessary for the operation of the Novasep Development Site and the Modified Novasep Facility and the manufacture and supply of Product at the Modified Novasep Facility in compliance with cGMP and Applicable Law (***"Facilities Licenses"***), as required for Novasep to perform the Development Services and its Product manufacturing and supply obligations under this Agreement. Novasep shall keep Celladon regularly informed about the status of all such Facilities Licenses and shall provide Celladon copies thereof upon request. Novasep shall ensure that the Modified Novasep Facility complies with cGMP and all other Applicable Law. Novasep shall use reasonable best efforts to resolve as soon as possible any issues that arise in its seeking or maintaining Facilities Licenses, including completely addressing and rectifying any deviations or other issues raised in any Warning Letter from the FDA or any similar warning or objection by the EMA or any other Regulatory Authority.

6.2 Compliance. In performing its obligations hereunder, Novasep shall comply with all Applicable Law and applicable requirements of Regulatory Authorities. Novasep shall obtain and maintain all government permits, including without limitation health, safety and environmental permits, necessary for the conduct of the actions and procedures undertaken to manufacture and supply Product during the Term. Without limiting the generality of the foregoing, Novasep shall comply with all regulatory requirements imposed by Applicable Law upon Novasep as the manufacturer of Product. Novasep shall, on a timely basis, provide Celladon with all information and documentation in Novasep's or its Affiliates' possession

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relevant to its role as the manufacturer of Product that is reasonably necessary for Celladon to comply with applicable regulatory requirements.

6.3 Cooperation and Assistance. Upon Celladon's written request, Novasep shall provide to Celladon all reasonable information, documentation and data in Novasep's or its Affiliates' possession relating to Product or its manufacture (or true and complete copies thereof) as Celladon may require for any purpose, including submissions to Regulatory Authorities in applications for Regulatory Approval or for the purpose of obtaining or maintaining Regulatory Approvals; in each case, at Celladon's expense for actual out-of-pocket copying costs and the costs (at a commercially reasonable FTE rate to be agreed by the parties in advance) for Novasep's internal employee time required for such copying. Without limiting the generality of the foregoing, upon Celladon's written request, Novasep shall provide to Celladon all documents specified in the Quality Agreement, including, by way of example only, the complete Master Batch Records and specific Manufacturing SOPs and updates as defined in the Quality Agreement, copies of executed, completed Batch Records for each Batch, and all relevant documents relating to the Manufacturing Process or any Novasep Project IP used in manufacturing Product.

6.4 Regulatory Approvals. Celladon (or its Affiliate or Licensee) shall have the exclusive right to prepare and submit any and all applications for Regulatory Approval for Product or Final Product, including any amendments or supplements thereto, and shall be responsible for obtaining and maintaining Regulatory Approvals. Any and all such applications and Regulatory Approvals for Product or Final Product shall be owned solely by and held in the name of Celladon (or its Affiliate or Licensee, as applicable). Novasep shall have no rights in or to any such applications or Regulatory Approvals.

6.5 Changes in Manufacturing Process. Novasep shall not change or modify the Manufacturing Process or any of the Manufacturing SOPs, or otherwise make any change in the materials, equipment, process or procedures used to manufacture or test Product that would require a filing with a Regulatory Authority and/or that would affect or reasonably be expected to affect Novasep's ability to manufacture the Product in accordance with the Specifications or the terms of this Agreement, or Celladon's ability to use Product supplied by Novasep in the production of Final Product for commercial distribution, in each case, without Celladon's prior written approval. No changes to Manufacturing Process, the Manufacturing SOPs, the Master Batch Records, material and in-process specifications and analytical procedures for raw materials, in-process testing or batch release testing shall be made except in accordance with the change control procedure set forth in the Quality Agreement. Novasep shall disclose all proposed changes in such manufacturing and testing materials, equipment, process or procedure to Celladon at a level that would be sufficient to allow Celladon to understand such changes and comply with applicable requirements of Regulatory Authorities. If Celladon agrees to allow any such change requiring Celladon's approval to be implemented, then the parties shall revise the Manufacturing SOPs and the relevant Specifications in writing accordingly, if applicable, in compliance with the requirements of the Quality Agreement. Any actual costs incurred by Novasep in making changes to the Manufacturing Process or the Manufacturing SOPs that are requested by Celladon shall be reimbursed by Celladon, in amounts to be agreed by the parties in writing prior to performing such changes.

6.6 Records. Novasep shall keep complete, accurate and authentic accounts, notes, data and records pertaining to the manufacture, processing, testing, storage, and distribution of the Product, including, without limitation, master production and control records, in accordance with Applicable Law and the requirements of Regulatory Authorities. Novasep shall retain such records for a period and in a manner consistent with applicable regulatory requirements and shall make available to Celladon copies of such records and shall permit Celladon to inspect the originals of such records as maintained by Novasep, on reasonable notice. After such time period, Novasep shall notify Celladon prior to the destruction of any records retained under this Section 6.6 and, at Celladon's request, shall transfer such records to Celladon.

6.7 Inspections by Celladon. Novasep shall permit Celladon to inspect the Novasep Facility during normal business hours and review such documents as is reasonably necessary for the purpose of assessing Novasep's compliance with the Manufacturing Process, the Manufacturing SOPs, cGMP, the Specifications, applicable requirements of Regulatory Authorities, and applicable manufacturing controls. Such inspection and document review shall be conducted upon reasonable prior written notice by Celladon prior to the proposed inspection, at a time and date mutually agreeable to the parties (except in the event of a reasonable, urgent concern by Celladon regarding the quality of Product, in which case Celladon may conduct the inspection with a prior reasonable prior written notice of only […***…] hours), and in accordance with the Quality Agreement. In addition, Celladon shall have the right to have a reasonable number of employees or agents present at the Novasep Facility during the preparation for or conduct of any manufacturing or production run for manufacture or packaging of a Batch of Product, and such employee or agent shall be free to inspect and oversee all aspects of such preparation or production run and to comment to Novasep thereupon. Celladon shall use its reasonable endeavors not to cause any undue disruption to Novasep's business and activity in carrying out such audit or inspection. For the avoidance of doubt, such inspection right of Celladon […***…].

6.8 Regulatory Inspections.

(a) Inspection by Regulatory Authorities. Upon the request of any Regulatory Authority having jurisdiction over the manufacture of Product hereunder, such Regulatory Authority shall have access to observe and inspect Novasep's facilities and procedures used for the manufacture, release and stability testing, and/or warehousing of all Product and to audit such facilities for compliance with cGMP and/or other applicable regulatory standards. Novasep specifically agrees to cooperate with any inspection by a Regulatory Authority, whether prior to or after Regulatory Approval of Product or Final Product, and to provide Celladon a copy of any inspection or audit report resulting from any such inspection. If Novasep is purchasing raw materials from a Third Party manufacturer for use in manufacturing Product, Novasep shall use Commercially Reasonable Efforts to ensure that such manufacturer's facilities and procedures are similarly subject to the provisions of this Section 6.8 as to the manufacture of such raw materials, and to ensure that Celladon is provided copies of any inspection or audit report of such Third Party relating to such raw materials.

(b) Notification of Inspections. Novasep agrees to notify Celladon within […***…] days of any written or oral inquiries, notifications or inspection activity by any Regulatory Authority in regard to Product supplied or to be supplied to Celladon hereunder. Novasep shall

provide a reasonable description of any such governmental inquiries, notifications or inspections promptly, but in no event later than […***…] days after such notification, inquiry or inspection. Novasep shall furnish to Celladon (i) within […***…] days after receipt, any report or correspondence issued by any Regulatory Authority in connection with such notification, inquiry or inspection, including any FDA Form 483 (List of Inspectional Observations) or applicable portions of any FDA Warning Letters which pertain to the Product (or any equivalent in another country or jurisdiction), and (ii) not later than […***…] days prior to the time it provides to any Regulatory Authority, copies of proposed responses or explanations relating to items set forth above (each, a ***"Proposed Response"***), in each case redacted of trade secrets or other confidential or proprietary information of Novasep that are unrelated to the obligations under this Agreement or are unrelated to Product or its manufacture. Novasep shall discuss with Celladon and consider in good faith any comments provided by Celladon on the Proposed Response. After the filing of a response with the FDA or other Regulatory Authority, Novasep shall notify Celladon of any further contacts with such Regulatory Authority relating to the subject matter of the response.

(d) Remedial Actions. Novasep shall notify Celladon immediately in writing in the event any action is taken or threatened by a Regulatory Authority relating to the manufacture or storage of Product by Novasep, or relating to the Novasep Facility in which such manufacture or storage occurs, or which may impair the ability of Novasep to manufacture Product (including any impairment to Novasep's ability to manufacture Product conforming to the applicable Specifications) in accordance with this Agreement. In any event, Novasep shall use best efforts to address and resolve as soon as possible any issues, concerns or warnings from any Regulatory Authority that might affect Novasep's ability to manufacture and supply Product in accordance with this Agreement. To the extent Novasep must implement a plan of remediation or for other modifications or changes to its Novasep Facility or its manufacturing processes in order to address and resolve any such issues, concerns or warnings from any Regulatory Authority, Novasep shall prepare such plan as soon as possible, shall provide a draft of the plan to Celladon for review and comment, and shall implement all reasonable comments of Celladon as soon as possible, and shall use Commercial Reasonable Efforts to implement and complete all aspects of the agreed plan as soon as possible.

6.9 Recalls. In the event Celladon shall be required or requested by any regulatory authority (or shall voluntarily decide in good faith) to recall any Final Product, Celladon shall coordinate such recall. If any such recall is or may be related to the Product incorporated in the recalled Final Product, Celladon shall immediately inform Novasep thereof. If a recall is due to Novasep's gross negligence, willful misconduct or breach of the Product Warranty by Novasep, and does not result from Celladon's gross negligence, willful misconduct or breach of this Agreement by Celladon, then Novasep shall reimburse Celladon for (i) the purchase price paid by Celladon to Novasep for the Product in such recalled Final Product, and (ii) Celladon's other reasonable and documented direct costs and expenses actually incurred by Celladon in connection with the recall, subject in any case to Sections 9.5 and 9.6 of this Agreement. If a recall is due to any reason other than Novasep's gross negligence, willful misconduct or breach of the Product Warranty, Celladon shall pay all of the costs and expenses of the recall.

6.10 Adverse Event Reporting. It is understood and agreed that Celladon (or its Affiliate or Licensee) shall have the sole right and responsibility for reporting any adverse events associated with Product or Final Product to the applicable and appropriate Regulatory

Authorities. Novasep shall provide Celladon all reasonable assistance in complying with such reporting requirements. Novasep shall notify Celladon, in accordance with the requirements of the Quality Agreement, of any information of which Novasep becomes aware concerning any side effect, injury, toxicity or sensitivity reaction, or any unexpected incident, and the severity thereof, that is associated with the manufacturing of Product.

7. FINANCIAL TERMS

7.1 Development Service Fees. Subject to Section 11.2 hereof, Celladon agrees to compensate Novasep for the performance of the services described in Article 3, the Technology Transfer Plan, the activities described in Article 6, and Modules 1, 2, 3, 4, 5, and 6 of the Scope of Work, as more fully described in any applicable Project Plans (collectively, the ***"Development Services"***) in an aggregate amount of up to […***…] Euro ([…***…] €). The fees relating to the Development Services includes both fees relating to Development Services as set forth in subsection (a), and reservations fees, as set forth in subsection (b), all payable as follows:

(a) <u>Development Services Fees</u>:

(i) two million and five hundred thousand Euro (2 500 000 €), which Novasep acknowledges has been paid in full prior to the Effective Date;

(ii) six hundred thousand Euro (600 000 €), to be paid no later than March 31, 2015;

(iii) eight hundred twenty five thousand Euro (825 000 €), to be paid no later than […***…], 2015;

(iv) eight hundred twenty five thousand Euro (825 000 €), to be paid no later than […***…], 2015;

(v) […***…] Euro ([…***…] €), to be paid no later than […***…];

(vi) […***…] Euro ([…***…] €), to be paid no later than […***…];

(vii) […***…] Euro ([…***…] €), to be paid no later than […***…];

(viii) […***…] Euro ([…***…] €), to be paid upon completion of […***…] scale, which is expected to take place no later than […***…]; such milestone will be deemed to be achieved upon […***…];

(ix) […***…] Euro ([…***…] €), to be paid no later than […***…];

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(x) […***…] Euro ([…***…] €), to be paid upon completion of the […***…], which is expected to take place no later than […***…]; such milestone will be deemed to be achieved upon […***…];

(xi) […***…] Euro ([…***…] €), to be paid upon completion of […***…], which is expected to take place no later than […***…]; such milestone will be deemed to be achieved upon […***…].

(xii) […***…] Euro ([…***…] €), to be paid no later than […***…];

(xiii) […***…] Euro ([…***…] €), to be paid upon […***…], which is expected to take place no later than […***…]; such milestone will be deemed to be achieved upon […***…];

(xiv) […***…] Euro ([…***…] €), to be paid no later than […***…].

(b) **Reservation Fees**: Celladon agrees to compensate Novasep for the reservation of manufacturing slots, in 2015 and 2016, for the Product in the Novasep Facility. As a consequence Celladon shall pay to Novasep an aggregate amount of […***…] Euro ([…***…] €), which shall be payable as follows:

(i) […***…] Euro ([…***…] €), to be paid no later than […***…];

(ii) […***…] Euro ([…***…] €), to be paid no later than […***…]

(c) For the sake of clarity, at completion of the Development Services, Celladon will have paid to Novasep (in accordance with Section 7.1 and Schedule 7.1):

(i) a total of […***…] thousand EUR ([…***…] €) in Development Services fees under Section 7.1(a) above; it being understood that such Development Services fees include, without limitation, the […***…]; and

(ii) the total amount of […***…] EUR ([…***…] €) as reservation fees for commercial production in the Novasep Facility for the first two calendar years of the Initial Term, under Section 7.1(b) above (such amount, the "***Additional Amount***"). The Additional Amount will be subject to recovery by Celladon in accordance with Section 7.2.

In addition, for the sake of further clarity, […***…] EUR ([…***…] €) of Novasep Facility Modifications Costs are included in the above mentioned aggregate amount of […***…] thousand EUR ([…***…] €), with such Novasep Facility Modifications Costs consisting of (i) […***…] Euro ([…***…] €) of Capital Expenditures as set forth in Schedule 7.1 and (ii) […***…] Euro ([…***…] €) to be financed in conformity with the payment schedule as defined in Schedule 7.1. Should however the Novasep Facility Modifications Costs be greater than […***…] EUR ([…***…] €), despite Novasep's efforts to mitigate these costs, then […***…].

7.2 Batch Prices. The per-Batch price to be paid by Celladon for a particular cGMP Batch of Product that is manufactured by Novasep during a particular calendar year and delivered to Celladon under this Agreement shall be as set forth below, subject to adjustment as set forth in Section 7.3:

Calendar Year	Price per Batch
2017	[…***…] EUR ([…***…] €)
2018	[…***…] EUR ([…***…] €)
2019	[…***…] EUR ([…***…] €)
2020	[…***…] EUR ([…***…] €)

Notwithstanding the foregoing, for Batches ordered during 2017 and 2018, the Batch Price listed above shall be reduced ratably by an aggregate of […***…] EUR ([…***…] €) in each such calendar year, across the minimum number of Batches applicable to such calendar year, as set forth in Schedule 7.1. If Celladon exercises the First Extension Option, Novasep shall invoice Celladon for, and Celladon shall pay, an advance payment equal to […***…]% of the total Batch Price for the minimum number of cGMP Batches for 2019 under Section 4.4(b), which will be deducted ratably from the Batch Price of each of the last […***…] ([…***…]) cGMP Batches purchased in 2019. If Celladon exercises the Second Extension Option, Novasep shall invoice Celladon for, and Celladon shall pay, an advance payment equal to […***…]% of the total Batch Price for the minimum number of cGMP Batches for 2020 under Section 4.4(b), which will be deducted ratably from the Batch Price of each of the last […***…] ([…***…]) cGMP Batches purchased in 2020.

In the event that this Agreement expires or is terminated before the Additional Amount, the 2019 advance payment or the 2020 advance payment (as applicable) has been applied in full to the purchase of cGMP Batches in the then-current Term, then Novasep shall deduct any unapplied portion of such payment from any other amounts due to Novasep as of such expiration or the effectiveness of such termination (as applicable), and if, after such deduction, any unapplied portion of such payment remains, Novasep shall promptly refund such unapplied portion to Celladon.

7.3 Adjustments to Batch Prices.

(a) The Batch Prices set forth in Section 7.2 have been defined based on the existing data and understanding of the production process for Product as of the Effective Date. Such Batch Prices may need to be revisited, and, if appropriate, modified, by the parties upon completion of the Development Services described in Sections 3.1, 3.2 and 3.3 if the efforts needed to produce one Batch are demonstrated in the course of such Development Services to be materially different from the parties' data and understanding as of the Effective Date. Any modification to the Batch Prices set forth in Section 7.2 would require that Novasep present appropriate data and documentation justifying the appropriateness of such modification and would be subject to mutual written agreement of the parties.

(b) Commencing on the first of December, 2017, and on the last month of each calendar year of the Term thereafter, the Batch Prices set forth in Section 7.2 (as modified in accordance with Section 7.3(a)) shall be adjusted, […***…] Any such price increase will be […***…]

(c) If the number of Batches subject to pending Purchase Orders and the Binding Period of the most recent Forecast will result in Celladon purchasing a number of Batches in a given year that exceeds the applicable minimum number of Batches under Section 4.4(a) or Section 4.4(b) for such year, the parties shall discuss in good faith appropriate reductions in the applicable Batch Price for such year.

(d) In the event that, as a result of any deviation from the Manufacturing Process by Novasep or its Affiliate (except with Celladon's express prior written approval) in the manufacture of any cGMP Batch that causes the yield from such manufacture to be less than a full Batch, the applicable Batch Price for such cGMP Batch as set forth in Section 7.2 (as adjusted pursuant to Section 7.3(a) and/or Section 7.3(b), as applicable) shall be reduced in proportion to such reduction in yield from the full Batch size.

7.4 Invoicing and Payment of Batch Price. Novasep shall provide to Celladon a written invoice for Product delivered in accordance with this Agreement, based on the then-applicable Batch Price under Section 7.2 (subject to adjustment in accordance with Section 7.3), provided that Novasep shall not deliver a written invoice for any Batch of Product before the Release of such Batch by Novasep. All amounts invoiced by Novasep pursuant to this Section 7.4 shall be payable on a net-30 basis, subject to Section 5.2.

7.5 Manner and Place of Payment. All payment amounts under this Agreement are expressed in Euro, and all payments hereunder shall be payable in Euro. All payments due by

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Celladon hereunder shall be made by bank wire transfer in immediately available funds to a bank and account designated in writing by Novasep.

7.6 Taxes. All prices in this Agreement exclude value added taxes (or VAT), U.S. state and local sales taxes, and other taxes (collectively hereinafter, "***Transaction Taxes***"), if any, imposed on the Products and services provided by Novasep under this Agreement.

8. INTELLECTUAL PROPERTY

8.1 Background IP.

(a) Celladon Background IP. Celladon shall at all times be and remain the sole and exclusive owner of all right, title and interest in and to Celladon Background IP. Novasep acknowledges and agrees that, as between Celladon and Novasep, all intellectual property rights in the Product are owned solely by Celladon. Neither Novasep nor any of its Affiliates shall acquire any rights of any kind whatsoever with respect to the Product as a result of this Agreement or the activities contemplated hereby.

(b) Novasep Background IP. Novasep shall at all times be and remain the sole and exclusive owner of all right, title and interest in and to the Novasep Background IP.

8.2 Project IP.

(a) Celladon Project IP. Celladon shall solely own, and Novasep hereby assigns to Celladon, all right, title and interest in and to all Celladon Project IP. Novasep shall cause any and all Information, inventions, developments and discoveries (whether or not patentable) within the Celladon Project IP to be collected and recorded in a timely, accurate, complete and professional manner sufficient for patent purposes. For clarity, however, notwithstanding Celladon's sole ownership of Celladon Project IP, Novasep's original laboratory notebooks in which any such Celladon Project IP is recorded or documented shall remain the property of Novasep. Novasep shall promptly disclose to Celladon in writing all Celladon Project IP arising under this Agreement. At Celladon's request and expense, Novasep shall provide Celladon with reasonable assistance to perfect Celladon's ownership interest in Celladon Project IP and in obtaining, securing and maintaining patents and other intellectual property rights therein. Novasep and all employees, agents, consultants and subcontractors of Novasep involved in the performance of the Services, shall sign and deliver to Celladon all writings and do all such things as may be necessary or appropriate to vest in Celladon all right, title and interest in and to Celladon Project IP. Celladon may, in its sole discretion, file and prosecute in its own name and at its own expense, patent applications on any patentable inventions within the Celladon Project IP. Upon the request of Celladon, and at the sole expense of Celladon, Novasep will assist Celladon in the preparation, filing and prosecution of such patent applications and will execute and deliver any and all instruments necessary to effectuate the ownership of such patent applications and to enable Celladon to file and prosecute such patent applications in any country.

(b) Novasep Project IP. Novasep shall solely own all right, title and interest in and to all Novasep Project IP.

8.3 Licenses.

(a) License to Novasep. Subject to the terms and conditions of this Agreement, Celladon hereby grants to Novasep during the Term – and where applicable, in particular for regulatory issues in connection with this Agreement, after the Term – […***…] for the […***…], for the […***…]. For clarity, as of the Effective Date, the […***…] specifically excludes […***…]. If the parties determine that a […***…], the parties will enter into a written amendment to this Agreement providing for the grant to Novasep and/or Henogen (as applicable) of a non-exclusive, non-transferable, royalty-free sublicense under such patent rights, subject to […***…].

(b) Licenses to Celladon.

(i) Novasep Project IP. Subject to the terms and conditions of this Agreement, Novasep hereby grants to Celladon a non-exclusive, worldwide, royalty-free, fully-paid, irrevocable, perpetual license, including the right to sublicense through multiple tiers of sublicense, under the Novasep Project IP, to make, have made, use, sell, have sold, offer for sale and import Product and Final Product, including, without limitation, to practice and use the Manufacturing Process in connection therewith.

(ii) Novasep Background IP. Should Novasep use any portion of Novasep Background IP in the manufacture of Product, Novasep shall, and it hereby does, grant to Celladon a non-exclusive, worldwide, royalty-free, fully-paid (except as expressly set forth below with respect to Patented Novasep Background IP), irrevocable, perpetual license, including the right to sublicense through multiple tiers of sublicense, under such portion of the Novasep Background IP, to make, have made, use, sell, have sold, offer for sale and import Product and Final Product; *provided, however,* that if Novasep uses any portion of the Novasep Background IP that is claimed by any issued patent owned or controlled by Novasep (***"Patented Novasep Background IP"***) in the manufacture of Product, before Novasep begins using such Patented Novasep Background IP in the manufacture of Product, the parties shall negotiate in good faith and mutually agree in writing upon commercially reasonable compensation to be paid by Celladon in the event that Celladon exercises its license under such Patented Novasep Background IP. If the parties are unable to reach mutual written agreement as to such compensation, then Novasep shall not use such Patented Novasep Background IP in the manufacture of Product. If Novasep uses any Patented Novasep Background IP in the manufacture of Product without first obtaining Celladon's written consent, then Novasep shall, and it hereby does, grant to Celladon a non-exclusive, worldwide, royalty-free, fully-paid, irrevocable, perpetual license, including the right to sublicense through multiple tiers of sublicense, under such Patented Novasep Background IP, to make, have made, use, sell, have sold, offer for sale and import Product and Final Product, including, without limitation, to practice and use the Manufacturing Process in connection therewith.

8.4 Technology Transfer. Celladon shall have the right, exercisable by written notice to Novasep at any time, to transfer the Manufacturing Process to itself, any of its Affiliates and/or any Third Party. Any such technology transfer pursuant to this Section 8.4 shall include the Master Batch Records, Manufacturing SOPs, Quality Control Procedures, the design of the Modified Novasep Facility, and all necessary portions of the Novasep Project IP used by Novasep in the manufacture of Product. If Celladon exercises its technology transfer right under this Section 8.4, Novasep shall provide reasonable technology transfer assistance services and access to documentation to Celladon as reasonably necessary to complete such technology transfer and enable Celladon, its Affiliate or its Third Party designee to replicate the Manufacturing Process as performed by Novasep, and Celladon shall pay Novasep for such services (at Novasep's then-standard rates) and reimburse Novasep for reasonable out-of-pocket expenses incurred in providing such services and access all as more fully detailed in a written technology transfer plan to be mutually agreed upon by the parties in good faith as promptly as practicable (and in any event within sixty days) after Celladon's exercise of such right.

8.5 No Implied Licenses. No right or license is granted under this Agreement by either party to the other party, either expressly or by implication, except those specifically set forth herein.

9. REPRESENTATIONS AND WARRANTIES

9.1 Mutual Representations and Warranties. Each party represents and warrants to the other that, as of the Effective Date: (a) it is duly organized and validly existing under the laws of its jurisdiction of incorporation or formation, and has full corporate or other power and authority to enter into this Agreement and to carry out the provisions hereof; (b) it is duly authorized to execute and deliver this Agreement and to perform its obligations hereunder, and the person or persons executing this Agreement on its behalf has been duly authorized to do so by all requisite corporate or partnership action; and (c) this Agreement is legally binding upon it, enforceable in accordance with its terms, and does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it may be bound, nor violate any material law or regulation of any court, governmental body or administrative or other agency having jurisdiction over it.

9.2 Celladon Representations and Warranties.

(a) Celladon Materials Warranty. Celladon represents and warrants to Novasep that the Celladon Materials delivered by or on behalf of Celladon to Novasep pursuant to this Agreement will conform, at the time of delivery to Novasep, to the Celladon Materials Specifications in effect at the time of such delivery (the ***"Celladon Materials Warranty"***).

(b) Intellectual Property. Celladon represents and warrants to Novasep as of the Effective Date that: (i) Celladon has the right to transfer to Novasep the Manufacturing Process and to disclose, transfer or make available to Novasep such existing Celladon Technology and other Confidential Information of Celladon as is necessary to permit Novasep to manufacture Product as contemplated by this Agreement; (ii) to Celladon's knowledge as of the Effective Date, the manufacture of Product using the Manufacturing Process does not infringe the issued patents or misappropriate any trade secrets or proprietary information of any Third Party; and (iii) Celladon has not received any written communication from any Third Party claiming that the

manufacture of Product using the Manufacturing Process infringes any intellectual property rights of any Third Party.

9.3 Novasep Representations and Warranties.

(a) Services Warranty. Novasep represents, warrants and covenants to Celladon that (i) the Development Services and the manufacture and supply of Product hereunder will be conducted by Novasep in a professional manner with professional skill and care and (ii) Novasep will use its good faith efforts to perform the Development Services in accordance with agreed timelines set forth in the Scope of Work, the Technology Transfer Plan and any other Project Plan. Celladon acknowledges that Novasep does not warrant that the Development Services will be successfully completed, that the results thereof will be acceptable to any Regulatory Authority to which they are presented, or that the Development Services will be completed within a specified time frame.

(b) Product Warranty. Novasep represents and warrants to Celladon that, at the time of delivery to Celladon, all Product delivered by Novasep hereunder: (i) will conform to the Specifications in effect at the date of manufacture; (ii) will have been manufactured in compliance with cGMP, the Quality Agreement, applicable Regulatory Approvals, and Applicable Laws, and in accordance with the Manufacturing Process as described in the Master Batch Records; and (iii) will be free and clear of any lien or encumbrance (collectively, the ***"Product Warranty"***).

(c) No Debarred or Disqualified Persons. Novasep represents and warrants to Celladon as of the Effective Date that neither Novasep nor any of its Affiliates, nor, to Novasep's knowledge, any of their respective employees, (i) is under investigation for debarment or is presently debarred by the FDA pursuant to 21 U.S.C. § 335a, or by the EMA or the Regulatory Authority of any European Union member state under any foreign equivalent thereof, or (ii) has a disqualification hearing pending or has been disqualified by the FDA pursuant to 21 C.F.R. § 312.70, or by the EMA or the Regulatory Authority of any European Union member state under any foreign equivalent thereof. Novasep further represents and warrants that neither it nor any of its Affiliates, nor, to Novasep's knowledge, any of their respective employees, has engaged in any conduct or activity which could lead to any of the above-mentioned disqualification or debarment actions. Novasep hereby covenants that neither Novasep nor any of its Affiliates will employ, contract with, or retain any person directly or indirectly to perform any services or other activities under this Agreement if such person (A) is under investigation for debarment or is presently debarred by the FDA, the EMA or the Regulatory Authority of any European Union member state, or (B) has a disqualification hearing pending or has been disqualified by the FDA, the EMA or the Regulatory Authority of any European Union member state. If, during the Term, Novasep, its Affiliate or any person employed or retained by Novasep or its Affiliate to perform any Development Services or any of the activities contemplated by Article 4 hereof (x) comes under investigation by the FDA, EMA or any other Regulatory Authority for a debarment action or disqualification, (y) is debarred or disqualified by the FDA, EMA or any other Regulatory Authority, or (z) engages in any conduct or activity that could lead to any of the above-mentioned disqualification or debarment actions, Novasep shall immediately notify Celladon of same, and Celladon shall have the right to terminate this Agreement immediately upon written notice to Novasep.

9.4 Novasep Responsibility for Performance by Affiliates. Celladon agrees that Novasep shall have the right to perform Development Services or Product manufacturing and supply activities hereunder through one or more Affiliates of Novasep, in particular Henogen; *provided,* in each case, that: (a) none of Celladon's rights hereunder are diminished or otherwise adversely affected as a result of such delegation; (b) each such Affiliate undertakes in writing obligations of confidentiality and non-use regarding Confidential Information of Celladon at least as stringent as those set forth in Article 10; and (c) Novasep shall at all times be fully responsible for the performance of such Affiliate and for the compliance of such Affiliate with this Agreement. The parties, via the POT or PGT, shall discuss any such proposed delegation reasonably in advance thereof, and Novasep shall consider any concerns raised by Celladon in good faith.

9.5 Disclaimer of Warranties. Except as expressly set forth in this Agreement, NEITHER PARTY MAKES ANY REPRESENTATIONS OR EXTENDS ANY WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, AND EACH PARTY EXPRESSLY DISCLAIMS ANY AND ALL WARRANTIES OF ANY KIND, EXPRESS OR IMPLIED, INCLUDING THE WARRANTIES OF DESIGN, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, NON-INFRINGEMENT OF THE INTELLECTUAL PROPERTY RIGHTS OF THIRD PARTIES, OR ARISING FROM A COURSE OF DEALING, USAGE OR TRADE PRACTICES.

9.6 Limitation of Liability. EXCEPT FOR LIABILITY FOR BREACH OF ARTICLE 10, NEITHER PARTY SHALL BE ENTITLED TO RECOVER FROM THE OTHER PARTY ANY SPECIAL, INDIRECT, INCIDENTAL, CONSEQUENTIAL OR PUNITIVE DAMAGES IN CONNECTION WITH THIS AGREEMENT; *provided, however,* that this Section 9.6 shall not be construed to limit either party's indemnification obligations under Article 12. FURTHER, NOVASEP'S LIABILITY UNDER THIS AGREEMENT WITH RESPECT TO ANY PURCHASE ORDER SHALL BE IN ANY CASE (EXCEPT FOR (A) ITS OBLIGATIONS UNDER SECTION 6.9, (B) BREACH OF ARTICLE 10 AND (C) THE GROSS NEGLIGENCE OR WILLFUL MISCONDUCT OF NOVASEP OR ITS AFFILIATES) LIMITED TO A TOTAL AMOUNT EQUAL TO THE […***…] FOR (I) […***…], (II) […***…] (III) […***…]DIRECTLY PRECEDING THE EVENT GIVING RISE TO CELLADON'S CLAIM, PROVIDED, HOWEVER THAT IF NO SUCH AMOUNTS LISTED ABOVE WERE PAID TO NOVASEP DURING SUCH[…***…]PERIOD, NOVASEP'S LIABILITY SHALL BE LIMITED TO […***…] EUR ([…***…] €). FOR THE AVOIDANCE OF DOUBT, CELLADON ACKNOWLEDGES AND AGREES THAT, EXCEPT FOR CELLADON'S RIGHTS TO TERMINATE THIS AGREEMENT UNDER ARTICLE 11, CELLADON'S SOLE REMEDY WITH RESPECT TO A NON-CONFORMING BATCH IS AS SET FORTH ABOVE IN SECTION 5.2 (EXCEPT IF SUCH NON-CONFORMING BATCH IS WHAT GIVES RISE TO A SUPPLY FAILURE).

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10. CONFIDENTIALITY

10.1 Confidentiality. Except to the extent expressly authorized by this Agreement, the Receiving Party agrees that, during the Term and for the applicable period thereafter specified in Section 10.7, it shall keep confidential and shall not publish or otherwise disclose to any Third Party, and shall not use for any purpose, except to the extent necessary to perform its obligations or to exercise its rights under this Agreement or as otherwise expressly provided for in this Agreement, any Confidential Information of the Disclosing Party. The Receiving Party shall use at least the same standard of care as it uses to protect proprietary or confidential information of its own (but in no event less than reasonable care) to ensure that neither the Receiving Party's nor any of its Affiliates' officers, directors, employees, consultants and agents (***"Representatives"***) disclose or make any unauthorized use of the Confidential Information, and the Receiving Party shall be liable for any breach of this Article 10 by any of its Representatives. The Receiving Party shall promptly notify the Disclosing Party upon discovery of any unauthorized use or unauthorized disclosure of the Disclosing Party's Confidential Information.

10.2 Exceptions. Confidential Information shall not include any Information which the Receiving Party can prove by competent evidence: (a) is now, or hereafter becomes, through no act or failure to act on the part of the Receiving Party in breach of this Agreement (or the Letter Agreement or Confidentiality Agreement, as applicable), generally known or available; (b) is known by the Receiving Party or any of its Affiliates at the time of receiving such Information from the Disclosing Party, as evidenced by its records (provided that the exception in this clause (b) shall not apply to Celladon Project IP); (c) is hereafter furnished to the Receiving Party or any of its Affiliates by a Third Party, as a matter of right and without restriction on disclosure; or (d) is independently discovered or developed by or on behalf of the Receiving Party and/or any of its Affiliates, without the use of Confidential Information of the Disclosing Party.

10.3 Authorized Disclosure. Notwithstanding Section 10.1, the Receiving Party may disclose Confidential Information, without violating its obligations under this Agreement, to the extent the disclosure is required by Applicable Law or by a valid order of a court or other governmental body of competent jurisdiction, provided that the Receiving Party, except where impracticable, gives reasonable prior written notice to the Disclosing Party of such required disclosure and, at the Disclosing Party's request and expense, cooperates with the Disclosing Party's efforts to obtain a protective order preventing or limiting the disclosure and/or requiring that the Confidential Information so disclosed be used only for the purposes for which the law or regulation requires, or for which the order was issued. In addition, each party shall have the right to disclose Confidential Information of the other party, including this Agreement, to Third Parties (including actual or *bona fide* potential investors, acquirers, or, in the case of Celladon, licensees, sublicensees, collaborators or other partners) in connection with due diligence or similar investigations by such Third Parties, and disclosure to potential Third Party investors in confidential financing documents; provided, in each case, that any such Third Party agrees to be bound by reasonable obligations of confidentiality and non-use.

10.4 Terms of this Agreement. Except as otherwise provided in this Article 10, each party agrees not to disclose to any Third Party the existence of this Agreement or the terms of

this Agreement without the prior written consent of the other party hereto, except as permitted by Section 10.3 or Section 10.5.

10.5 Public Announcements.

(a) Except as required by applicable law, rule or regulation (including disclosure requirements of the U.S. Securities and Exchange Commission (***"SEC"***) or any stock exchange on which securities issued by a party or its Affiliates are traded), neither party shall make any public announcement concerning this Agreement or the subject matter hereof without the prior written consent of the other, which shall not be unreasonably withheld or delayed; provided that each party may make any public statement in response to questions by the press, analysts, investors or those attending industry conferences or financial analyst calls, or issue press releases, so long as any such public statement or press release is not inconsistent with prior public disclosures or public statements approved by the other party pursuant to this Section 10.5 and which do not reveal non-public information about the other party. In the event of a required public announcement, to the extent practicable under the circumstances, the party making such announcement shall provide the other party with a copy of the proposed text of such announcement sufficiently in advance of the scheduled release to afford such other party a reasonable opportunity to review and comment upon the proposed text.

(b) The parties shall coordinate in advance with each other in connection with the disclosure or filing of this Agreement (including redaction of certain provisions of this Agreement) with the SEC, any stock exchange on which securities issued by a party or its Affiliate are traded, or any other governmental authority, and each party shall use Commercially Reasonable Efforts to seek confidential treatment for the terms proposed to be redacted, provided that each party shall ultimately retain control over what information to disclose to the SEC, any such stock exchange or any governmental authority. Other than such obligation, neither party (nor its Affiliates) shall be obligated to consult with or obtain approval from the other party with respect to any filings SEC, any such stock exchange or any governmental authority.

10.6 Return of Confidential Information. Upon termination or expiration of the Agreement, or upon written request of the Disclosing Party, a Receiving Party will promptly return to the Disclosing Party or destroy all documents, notes and other tangible materials comprising or containing the Disclosing Party's Confidential Information and all copies thereof; *provided, however,* that each party may retain a copy of the other party's Confidential Information for the purpose of monitoring compliance with its obligations under this Agreement, exercising the rights or licenses expressly granted to such party under this Agreement (for so long as such rights or licenses are in effect) or as required by Applicable Law. Further, the foregoing return or destruction requirement shall not apply to electronic copies of files created automatically in the ordinary course of business pursuant to the Receiving Party's standard electronic back-up and archival procedures so long as such electronic files are (a) maintained only on centralized storage servers (and not on personal computers or devices), and (b) not readily accessible by the Receiving Party's personnel (other than its information technology specialists).

10.7 Term of Confidentiality Obligations. The Receiving Party's obligations under this Article 10 shall survive expiration or any termination of this Agreement.

11. TERM AND TERMINATION

11.1 Term. The term of this Agreement (the ***"Term"***) shall commence on the Collaboration Initiation Date and, unless earlier terminated in accordance with this Article 11 or Section 9.3(c), shall expire on December 31, 2018 (the "***Initial Term***"), provided that, subject to Section 4.4(b), Celladon shall have the option, exercisable at Celladon's sole discretion, to extend the expiration of the Term until:

(a) December 31, 2019 (the ***"First Extension Option"***), exercisable by delivery of written notice thereof to Novasep no later than […***…]; and

(b) Provided that Celladon exercises the First Extension Option, December 31, 2020 (the ***"Second Extension Option"***), exercisable by delivery of written notice thereof to Novasep no later than […***…].

11.2 Early Go/No-Go Decisions. The parties acknowledge that a critical development event regarding the Product, expected to occur in the first half of 2015, could substantially impact the viability of the Project and this Agreement, and agree as set forth in this Section 11.2. If, based on the unblinding of the results of Celladon's Phase 2b clinical trial of Mydicar described in Celladon Protocol No. CELL-004, titled "A Phase 2b, Double-Blind, Placebo-Controlled, Multinational, Multicenter, Randomized Study Evaluating the Safety and Efficacy of Intracoronary Administration of MYDICAR® (AAV1/SERCA2a) in Subjects With Heart Failure" (the ***"CUPID 2 Data"***), Celladon concludes in good faith that the CUPID 2 Data is such that Celladon does not require production of Product at the Novasep Facility, Celladon shall have the right to terminate this Agreement upon written notice to Novasep delivered no later than […***…], 2015. In the event of such termination, Novasep shall be entitled to retain the amounts paid by Celladon to Novasep pursuant to Sections 7.1(a)(i), 7.1(a)(ii), 7.1(a)(iii) and 7.1(a)(iv) of this Agreement prior to such termination (and Celladon shall be obligated to pay any such amount that has become due pursuant to Section 7.1(a)(ii), 7.1(a)(iii) or 7.1(a)(iv) of this Agreement but has not previously been paid), but all other rights and obligations of the parties under this Agreement including any obligation of Celladon to pay the amounts set forth in Section 7.1 of this Agreement and **Schedule 7.1** hereto, shall terminate, subject only to Section 11.6 of this Agreement.

11.3 Termination for Material Breach.

(a) A party may terminate this Agreement for material breach of this Agreement by the other party upon […***…] days' written notice specifying the nature of the breach, if such material breach has not been cured within such […***…]-day period.

(b) At any time after validation of the Manufacturing Process at the Novasep Facility, Celladon may terminate this Agreement upon written notice to Novasep if Novasep suspends production of Product at the Novasep Facility or if operation of the Novasep Facility is otherwise shut down, in each case, for more than […***…] ([…***…]) consecutive months for any reason, such as, by way of example and not limitation, (i) damage to, destruction of, or other failure of the Novasep Facility, (ii) failure of the equipment used to manufacture Product at the Novasep Facility, (iii) termination or revocation of Facility License(s), or (iv) suspension due to receipt by

Novasep or its Affiliate of any FDA Warning Letter or similar warning or objection by the EMA or any other Regulatory Authority and failure by Novasep or its Affiliate to completely rectify to the applicable Regulatory Authority's satisfaction all violations identified therein), unless such suspension or shut down are the sole and direct result of Celladon's negligence, willful misconduct or breach of this Agreement.

(c) Should Celladon terminate this Agreement in accordance with Section 11.3(a) due to uncured material breach by Novasep (but not for termination under Section 11.3(b)), Celladon shall […***…].

11.4 Termination for Insolvency. Each party may terminate this Agreement upon fifteen days prior written notice to the other party if the other party (a) files in any court or agency, pursuant to any statute or regulation of any state or country, a petition in bankruptcy or insolvency or for reorganization or for the appointment of a receiver or trustee of such other party or of substantially all of its assets, (b) proposes a written agreement of composition or extension of substantially all of its debts, (c) is served with an involuntary petition against it, filed in any bankruptcy or insolvency proceeding, and such petition is not dismissed within 90 days after the filing thereof, (d) proposes or is a party to any dissolution or liquidation, or (e) makes an assignment of substantially all of its assets for the benefit of its creditors. Should Celladon terminate this Agreement in accordance with Section 11.4, Celladon shall pay to Novasep the Batch Price of all quantities of Product ordered by Celladon and already manufactured but not yet delivered, under production or under Purchase Orders submitted by Celladon, and Novasep shall deliver such quantities of Product to Celladon in accordance with this Agreement.

11.5 Termination by Celladon for Other Reasons. Celladon shall have the right to terminate this Agreement at any time for reasons other than those stated in Sections 11.2, 11.3 and 11.4 above, or for its convenience, upon written notice to Novasep delivered no later than March 31, 2016, subject to Section 14.7. If Celladon delivers written notice of termination of this Agreement pursuant to this Section 11.5 on or before March 31, 2016 (or such later time as is permitted under Section 14.7), Novasep shall be entitled to retain all amounts paid by Celladon to Novasep pursuant to Section 7.1 of this Agreement and **Schedule 7.1** hereto prior to the date such notice of termination is delivered, and Celladon shall pay to Novasep:

(a) […***…]; and

(b) a termination fee of […***…] EUR ([…***…] €).

11.6 Accrued Obligations; Survival. Neither expiration nor any termination of this Agreement shall relieve either party of any obligation or liability accruing prior to such

expiration or termination, nor shall expiration or any termination of this Agreement preclude either party from pursuing all rights and remedies it may have under this Agreement, at law or in equity, with respect to breach of this Agreement. In addition, the parties' rights and obligations under Sections 6.3, 6.6, 6.8, 6.9, 6.10, 7.2 (last paragraph only), 7.6, 9.5, 9.6, 10.1, 10.2, 10.3, 10.4, 10.6, 10.7, 11.2, 11.5 and 11.6 and Articles 8, 12, 13 and 14 of this Agreement shall survive expiration or any termination of this Agreement.

12. INDEMNIFICATION

12.1 Celladon Indemnification. Celladon hereby agrees to save, defend, indemnify and hold harmless Novasep, its Affiliates, and its and their respective officers, directors, employees, consultants and agents (***"Novasep Indemnitees"***) from and against any and all losses, damages, liabilities, expenses and costs, including reasonable legal expense and attorneys' fees (***"Losses"***), to which any such Novasep Indemnitee may become subject as a result of any claim, demand, action or other proceeding by any Third Party to the extent such Losses arise out of: (a) the gross negligence or willful misconduct of any Celladon Indemnitee (defined below); or (b) the development, manufacture, use, handling, storage, sale or other disposition of Product or Final Product by or on behalf of Celladon or any of its Affiliates or Third Party licensees or sublicensees; except, in each case, to the extent such Losses result from the gross negligence or willful misconduct of any Novasep Indemnitee.

12.2 Novasep Indemnification. Novasep hereby agrees to save, defend, indemnify and hold harmless Celladon, its Affiliates, and its and their respective officers, directors, employees, consultants, contractors and agents (***"Celladon Indemnitees"***) from and against any and all Losses to which any such Celladon Indemnitee may become subject as a result of any claim, demand, action or other proceeding by any Third Party to the extent such Losses arise out of the gross negligence or willful misconduct of any Novasep Indemnitee; except, in each case, to the extent such Losses result from the gross negligence or willful misconduct of any Celladon Indemnitee.

12.3 Indemnification Procedures. In the event a party (the ***"Indemnified Party"***) seeks indemnification under Section 12.1 or Section 12.2, it shall inform the other party (the ***"Indemnifying Party"***) of a Claim as soon as reasonably practicable after it receives notice of the Claim (it being understood and agreed, however, that the failure by an Indemnified Party to give notice of a claim as provided in this Section 12.3 shall not relieve the Indemnifying Party of its indemnification obligations under this Agreement except and only to the extent that such Indemnifying Party is actually damaged as a result of such failure to give notice), shall permit the Indemnifying Party to assume direction and control of the defense of the Claim (including the right to settle the Claim solely for monetary consideration) using counsel reasonably satisfactory to the Indemnified Party, and shall cooperate as requested (at the expense of the Indemnifying Party) in the defense of the Claim. The Indemnified Party may participate in the defense of the Claim at its own expense. The Indemnifying Party shall keep the Indemnified Party advised of the status of such action, suit, proceeding or claim and the defense thereof. The Indemnified Party shall not agree to any settlement of such action, suit, proceeding or claim without the prior written consent of the Indemnifying Party, which shall not be unreasonably withheld, delayed or conditioned. The Indemnifying Party shall not agree to any settlement of such action, suit, proceeding or claim or consent to any judgment in respect thereof that does not

include a complete and unconditional release of the Indemnified Party from all liability with respect thereto, that imposes any liability or obligation on the Indemnified Party or that acknowledges fault by the Indemnified Party without the prior written consent of the Indemnified Party.

13. DISPUTE RESOLUTION

13.1 Disputes. Subject to Section 13.3, any claim, dispute, or controversy as to the breach, enforcement, interpretation or validity of this Agreement (each, a ***"Dispute"***) will be referred to the Chief Executive Officer of Celladon and the Chief Executive Officer of Novasep for attempted resolution. In the event such individuals are unable to resolve such Dispute within 30 days of such Dispute being referred to them, then, upon the written request of either party to the other party, the Dispute shall be subject to arbitration in accordance with Section 13.2, except as expressly set forth in Section 13.3.

13.2 Arbitration.

(a) Claims. Subject to Section 13.3 below, any Dispute that is not resolved under Section 2.3 or Section 13.1, as applicable, within the applicable period shall be resolved by final and binding arbitration administered by the International Chamber of Commerce (the ***"Administrator"***) in accordance with its then-effective comprehensive arbitration rules and procedures (the ***"Rules"***), except to the extent any such Rule conflicts with the express provisions of this Section 13.2. (Capitalized terms used but not otherwise defined in this Agreement shall have the meanings provided in the Rules.). The IBA Rules on the Taking of Evidence in International Arbitration and the IBA Rules of Ethics for International Arbitrators shall be utilized and applied in the Arbitration. The Arbitration shall be conducted by one neutral arbitrator selected in accordance with the Rules, provided that such individual shall not have any conflict of interest or be a current or former employee or director, or a current stockholder, of either party or any of their respective Affiliates. The arbitration and all associated proceedings and communications shall be conducted in English, and the arbitration shall be held in San Diego, California, USA, if Novasep makes the written request for arbitration pursuant to Section 2.3 or Section 13.1 (as applicable), and in London, England, if Celladon makes the written request for arbitration pursuant to Section 2.3 or Section 13.1 (as applicable).

(b) Hearing; Decision. The Arbitrator shall require that each party submit concise written statements of position and shall permit the submission of rebuttal statements, subject to reasonable limitations on the length of such statements to be established by the Arbitrator. The Hearing shall be no longer than five business days in duration. The Arbitrator shall also permit the submission of expert reports. The Arbitrator shall render the Award within 30 days after the Arbitrator declares the Hearing closed, and the Award shall include a written statement describing the essential findings and conclusions on which the Award is based, including the calculation of any damages awarded. The Arbitrator will, in rendering his or her decision, apply the substantive law of the State of New Jersey, USA, excluding its conflicts of laws principles, however the law of the arbitration will be that of the jurisdiction in which the arbitration is being held (i.e., California or England, as applicable). The Arbitrator's authority to award damages shall be subject to the limitations set forth in Section 9.6. The Award rendered

by the Arbitrator shall be final, binding and non-appealable, and judgment may be entered upon it in any court of competent jurisdiction.

(c) Costs. Each party shall bear its own attorney's fees, costs, and disbursements arising out of the arbitration, and shall pay an equal share of the fees and costs of the arbitrator and the Administrator; *provided, however,* the Arbitrator shall be authorized to determine whether a party is the prevailing party, and if so, to award to that prevailing party reimbursement for any or all of its reasonable attorneys' fees, costs and disbursements (including, for example, expert witness fees and expenses, photocopy charges, travel expenses, *etc.*), and/or the fees and costs of the Administrator and the Arbitrator.

13.3 Court Actions. Nothing contained in this Agreement shall deny either party the right to seek injunctive or other equitable relief from a court of competent jurisdiction in the context of a *bona fide* emergency or prospective irreparable harm, and such an action may be filed and maintained notwithstanding any ongoing discussions between the parties or any ongoing arbitration proceeding. In addition, either party may bring an action in any court of competent jurisdiction to resolve disputes pertaining to the validity, construction, scope, enforceability, infringement or other violations of patent or other intellectual property rights, and no such claim shall be subject to arbitration pursuant to Section 13.2. Each of the Parties herby waives to the fullest extent permitted by Applicable Law any right it may have to a trial by jury with respect to any litigation directly or indirectly arising out of, under or in connection with this Agreement or the transactions contemplated by this Agreement. Each of the Parties hereby (a) certifies that no representative, agent or attorney of the other Party has represented, expressly or otherwise, that such other Party would not, in the event of litigation, seek to enforce the foregoing waiver and (b) acknowledges that it has been induced to enter into this Agreement and the transactions contemplated by this Agreement, as applicable, by, among other things, the mutual waivers and certifications in this Section.

14. MISCELLANEOUS

14.1 Relationship between the Parties. The parties' relationship, as established by this Agreement, is solely that of independent contractors. This Agreement does not create any partnership, joint venture or similar business relationship between the parties. Neither party is a legal representative of the other party, and neither party may assume or create any obligation, representation, warranty or guarantee, express or implied, on behalf of the other party for any purpose whatsoever.

14.2 Governing Law. This Agreement shall be governed by and construed in accordance with the laws of the State of New Jersey, USA, excluding its conflicts of laws principles.

14.3 Entire Agreement; Amendments. This Agreement (including the Exhibits hereto) is both a final expression of the parties' agreement and a complete and exclusive statement with respect to all of its terms. This Agreement supersedes all prior and contemporaneous agreements and communications, whether oral, written or otherwise, concerning any and all matters contained herein, including the Letter Agreement and the Confidentiality Agreement. The Exhibits to this Agreement are incorporated herein by reference

and shall be deemed a part of this Agreement. This Agreement may be amended, or any term hereof modified, only by a written instrument duly executed by authorized representatives of both parties hereto.

14.4 Non-Waiver. The failure of a party to insist upon strict performance of any provision of this Agreement or to exercise any right arising out of this Agreement shall neither impair that provision or right nor constitute a waiver of that provision or right, in whole or in part, in that instance or in any other instance. Any waiver by a party of a particular provision or right shall be in writing, shall be as to a particular matter and, if applicable, for a particular period of time and shall be signed by such party.

14.5 Approval of Subcontractors. Novasep shall not subcontract any of the Development Services or Product manufacturing and supply activities hereunder to any Third Party without the prior written consent of Celladon or the prior approval of the PGT, except to the extent such subcontracting is expressly contemplated by, and the Third Party contractor is identified in, the Scope of Work, the Technology Transfer Plan, any Project Plan or the Quality Agreement. To the extent Novasep is permitted to subcontract Development Services or Product manufacturing and supply activities hereunder to a Third Party contractor pursuant to the preceding sentence, Novasep may do so; *provided*, in each case, that: (a) none of Celladon's rights hereunder are diminished or otherwise adversely affected as a result of such subcontracting; (b) each such Third Party contractor undertakes in writing obligations of confidentiality and non-use regarding Confidential Information of Celladon at least as stringent as those set forth in Article 10 and obligations with respect to assignment of work product and intellectual property rights resulting from subcontracted activities sufficient for Novasep to comply with its obligations under Article 8; and (c) Novasep shall be fully responsible for the compliance of each such Third Party contractor with all applicable terms and conditions of this Agreement and for payment of such Third Party contractor.

14.6 Assignment. Except as expressly provided hereunder, neither this Agreement nor any rights or obligations hereunder may be assigned or otherwise transferred by either party without the prior written consent of the other party (which consent shall not be unreasonably withheld); *provided, however,* that either party may assign this Agreement and its rights and obligations hereunder without the other party's consent: (a) in connection with the transfer or sale of all or substantially all of the business of such party to which this Agreement relates to a Third Party, whether by merger, sale of stock, sale of assets or otherwise; or (b) to an Affiliate, provided that the assigning party shall remain liable and responsible to the non-assigning party hereto for the performance and observance of all such duties and obligations by such Affiliate. The rights and obligations of the parties under this Agreement shall be binding upon and inure to the benefit of the successors and permitted assigns of the parties, and the name of a party appearing herein will be deemed to include the name of such party's successors and permitted assigns to the extent necessary to carry out the intent of this section. Any assignment not in accordance with this Agreement shall be void.

14.7 Force Majeure. Each party shall be excused from liability for the failure or delay in performance of any obligation under this Agreement by reason of any event beyond such party's reasonable control, including but not limited to Acts of God, fire, flood, explosion, earthquake, or other natural forces, war, civil unrest, acts of terrorism, accident, destruction or

other casualty, any lack or failure of transportation facilities, any lack or failure of supply of raw materials, any strike or labor disturbance, or any other event similar to those enumerated above. Such excuse from liability shall be effective only to the extent and duration of the event(s) causing the failure or delay in performance and provided that the party has not caused such event(s) to occur. The affected party shall notify the other party of such force majeure circumstances as soon as reasonably practical, and shall promptly undertake all Commercially Reasonable Efforts necessary to cure such force majeure circumstances. Notwithstanding the foregoing:

(a) should any force majeure circumstances affecting Novasep's performance of its obligations under this Agreement continue for longer than […***…] ([…***…]) months, then Celladon shall be excused from its obligation to pay the Take or Pay Compensation until such time as such force majeure circumstances are cured; and

(b) should any such force majeure circumstances continue for longer than […***…] ([…***…]) months, Celladon shall have the right to terminate this Agreement under and in accordance with Section 11.5, except that the termination fee due under Section 11.5(b) shall in such case be […***…] the amount specified therein.

14.8 Severability. If any one or more of the provisions contained in this Agreement is held invalid, illegal or unenforceable in any respect, the validity, legality and enforceability of the remaining provisions contained herein shall not in any way be affected or impaired thereby, unless the absence of the invalidated provision(s) adversely affects the substantive rights of the parties. The parties shall in such an instance use their best efforts to replace the invalid, illegal or unenforceable provision(s) with valid, legal and enforceable provision(s) which, insofar as practical, implement the purposes of this Agreement.

14.9 Notices. All notices which are required or permitted hereunder shall be in writing and sufficient if delivered personally, sent by facsimile (and promptly confirmed by personal delivery, registered or certified mail or express courier), sent by internationally-recognized express courier or sent by mail, postage prepaid, addressed as follows:

In the case of Celladon:

Celladon Corporation
11988 El Camino Real, Suite 650
San Diego, CA 92130-3579
USA
Fax: +1 (858) 964-0974
Attention: President and Chief Financial Officer

With a required copy to:

Celladon Corporation
11988 El Camino Real, Suite 650
San Diego, CA 92130-3579
USA
Fax: +1 (858) 964-0974
Attention: General Counsel

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In the case of Novasep:

Novasep, Inc.
23 Creek Circle
Boothwyn PA 19061
USA
Fax : +1
Attention: C.E.O.

With a required copy to:

Novasep Holding SAS
39, rue Saint Jean de Dieu
F-69007 Lyon (France)
France
Attention : Chief Legal Officer

or to such other address(es) as the party to whom notice is to be given may have furnished to the other party in writing in accordance herewith. Any such notice shall be deemed to have been given: (a) when delivered, if personally delivered or sent by facsimile on a business day (or if delivered or sent on a non-business day, then on the next business day); (b) on the business day after dispatch, if sent by internationally-recognized express courier; or (c) on the fourth (4^{th}) Business Day following the date of mailing, if sent by mail.

14.10 Interpretation. The headings of clauses contained in this Agreement preceding the text of the sections, subsections and paragraphs hereof are inserted solely for convenience and ease of reference only and shall not constitute any part of this Agreement, or have any effect on its interpretation or construction. All references in this Agreement to the singular shall include the plural where applicable. The term "including" or "includes" as used in this Agreement means including, without limiting the generality of any description preceding such term, and the word "or" has the inclusive meaning represented by the phrase "and/or." Unless otherwise specified, references in this Agreement to any section shall include all subsections and paragraphs in such Section and references in this Agreement to any subsection shall include all paragraphs in such subsection. All references to days in this Agreement shall mean calendar days, unless otherwise specified. Ambiguities and uncertainties in this Agreement, if any, shall not be interpreted against either party, irrespective of which party may be deemed to have caused the ambiguity or uncertainty to exist. This Agreement has been prepared in the English language, and the English language shall control its interpretation. In addition, all notices required or permitted to be given hereunder, and all written, electronic, oral or other communications between the parties regarding this Agreement shall be in the English language.

14.11 Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed an original document, and all of which, together with this writing, shall be deemed one instrument. This Agreement may be executed by facsimile or PDF signatures, which signatures shall have the same force and effect as original signatures.

[Signature page follows]

IN WITNESS WHEREOF, the parties hereto have duly executed this Development, Manufacturing and Supply Agreement as of the Effective Date.

CELLADON CORPORATION

By: /s/ Paul B. Cleveland
Name: Paul B. Cleveland
Title: President and CFO
Date: March 20, 2015

NOVASEP, INC.

By: /s/ Andrew Brennan
Name: Andrew Brennan
Title: C.E.O.
Date: March 20, 2015

Exhibit Index:

All the following appendixes and exhibits are attached to this Agreement and form an integral part thereof :

Exhibit A	Celladon Materials Specifications
Exhibit B	Scope of Work
Exhibit C	Specifications
Exhibit D	Quality Agreement
Exhibit E	Price table

Exhibit B

[...***...]

***Confidential Treatment Requested

Exhibit E

[…***…]

***Confidential Treatment Requested

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the Registration Statement (Form S-8 No. 333-193662) pertaining to the 2001 Stock Option Plan, the 2012 Equity Incentive Plan, the 2013 Equity Incentive Plan and the 2013 Employee Stock Purchase Plan of Celladon Corporation of our report dated March 31, 2015, with respect to the consolidated financial statements of Celladon Corporation included in this Annual Report (Form 10-K) for the year ended December 31, 2014.

/s/ Ernst & Young LLP

San Diego, California
March 31, 2015

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO RULE 13a-14(a) AND 15d-14(a) OF THE SECURITIES EXCHANGE ACT, AS ADOPTED PURSUANT
TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Krisztina M. Zsebo, Ph.D., certify that:

1. I have reviewed this Annual Report on Form 10-K of Celladon Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f) for the registrant and have:

a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 31, 2015

/s/ Krisztina M. Zsebo, Ph.D.
Krisztina M. Zsebo, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO RULE 13a-14(a) AND 15d-14(a) OF THE SECURITIES EXCHANGE ACT, AS ADOPTED PURSUANT
TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Paul B. Cleveland, certify that:

1. I have reviewed this Annual Report on Form 10-K of Celladon Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f) for the registrant and have:

a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 31, 2015

/s/ Paul B. Cleveland
Paul B. Cleveland
President and Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Krisztina M. Zsebo, Ph.D., Chief Executive Officer of Celladon Corporation (the "Registrant"), do hereby certify in accordance with 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, based upon my knowledge:

(1) this Annual Report on Form 10-K of the Registrant, to which this certification is attached as an exhibit (the "Report"), fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m); and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Registrant.

Date: March 31, 2015

/s/ Krisztina M. Zsebo, Ph.D.
Krisztina M. Zsebo, Ph.D.
Chief Executive Officer

This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities Exchange Commission and is not incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.

Exhibit 32.2

CERTIFICATION PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Paul B. Cleveland, President and Chief Financial Officer of Celladon Corporation (the "Registrant"), do hereby certify in accordance with 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, based upon my knowledge:

(1) this Annual Report on Form 10-K of the Registrant, to which this certification is attached as an exhibit (the "Report"), fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m); and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Registrant.

Date: March 31, 2015

/s/ Paul B. Cleveland
Paul B. Cleveland
President and Chief Financial Officer

This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities Exchange Commission and is not incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.