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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2015

Or

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

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)**

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

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 Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 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 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. (Check one):

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 number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of April 30, 2015 was 23,907,212.

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CELLADON CORPORATION

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FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2015

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

Celladon Corporation

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

	March 31, 2015 (unaudited)	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 50,018	$ 14,435
Short-term investments	20,570	70,513
Prepaid expenses and other assets	1,673	3,135
Total current assets	72,261	88,083
Property and equipment, net	793	763
Other assets	185	264
Total assets	$ 73,239	$ 89,110
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 3,746	$ 5,803
Accrued clinical expenses	646	731
Accrued interest	71	71
Current portion of long-term obligations	1,671	1
Total current liabilities	6,134	6,606
Long-term obligations, net of discount	8,710	10,102
Non-current liabilities	291	298
Commitments and contingencies (Note 5)		
Stockholders' equity:		
Preferred stock, $0.001 par value; authorized shares — 10,000,000 at March 31, 2015 and December 31, 2014, respectively; no shares issued and outstanding	—	—
Common stock, $0.001 par value; authorized shares — 200,000,000 at March 31, 2015 and December 31, 2014, respectively; issued and outstanding — 23,827,918 and 23,490,737 at March 31, 2015 and December 31, 2014, respectively	24	23
Additional paid-in capital	221,266	218,528
Accumulated other comprehensive loss	(1)	(8)
Accumulated deficit	(163,185)	(146,439)
Total stockholders' equity	58,104	72,104
Total liabilities and stockholders' equity	$ 73,239	$ 89,110

See accompanying notes.

Celladon Corporation

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

	Three Months Ended March 31,	
	2015	2014
Operating expenses:		
Research and development	$ 11,518	$ 5,218
General and administrative	4,779	1,706
Total operating expenses	16,297	6,924
Loss from operations	(16,297)	(6,924)
Other income (expense):		
Interest income	40	8
Interest expense	(510)	(59)
Other income (expense)	21	(4)
Change in fair value of warrant liability	—	(183)
Consolidated net loss	(16,746)	(7,162)
Other comprehensive gain (loss):		
Unrealized gain (loss) on investments	7	(2)
Comprehensive loss	$ (16,739)	$ (7,164)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.71)	$ (0.60)
Weighted-average shares outstanding, basic and diluted	23,667,800	11,939,866

See accompanying notes.

Celladon Corporation

Consolidated Statements of Cash Flows
(in thousands)
(Unaudited)

	Three Months Ended March 31,	
	2015	2014
Cash flows from operating activities		
Consolidated net loss	$(16,746)	$ (7,162)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation	76	26
Stock-based compensation	2,103	519
Noncash interest expense	301	59
Amortization of investment premium	148	13
Change in fair value of warrant liability	—	183
Gain on disposal of property and equipment	(1)	—
Deferred rent	(4)	(1)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	1,518	(403)
Accounts payable and accrued expenses	(2,160)	219
Net cash used in operating activities	(14,765)	(6,547)
Cash flows from investing activities		
Purchases of investment securities	—	(250)
Proceeds from maturities of investment securities	49,802	6,950
Purchases of property and equipment	(94)	(212)
Proceeds from sale of property and equipment	4	—
Net cash provided by investing activities	49,712	6,488
Cash flows from financing activities		
Proceeds from issuance of common stock	636	50,600
Costs paid in connection with common stock offering	—	(4,567)
Net cash provided by financing activities	636	46,033
Net increase in cash and cash equivalents	35,583	45,974
Cash and cash equivalents, beginning of period	14,435	7,903
Cash and cash equivalents, end of period	$ 50,018	$53,877

See accompanying notes.

Celladon Corporation

Notes to Consolidated Financial Statements

(Unaudited)

1. Basis of Presentation, Organization and Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited consolidated financial statements of Celladon Corporation (Celladon or the Company) should be read in conjunction with the audited financial statements and notes thereto as of and for the year ended December 31, 2014 included in the Company's Annual Report on Form 10-K (Annual Report) filed with the Securities and Exchange Commission (SEC). The accompanying financial statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial information and in accordance with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, since they are interim statements, the accompanying financial statements do not include all of the information and notes required by GAAP for complete financial statements. In the opinion of management, the accompanying financial statements reflect all adjustments (consisting of normal recurring adjustments) that are necessary for a fair statement of the financial position, results of operations and cash flows for the interim periods presented. Interim results are not necessarily indicative of results for a full year. 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 and assumptions.

Organization

Celladon 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.

As of March 31, 2015, 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.

Principles of Consolidation

The financial statements of the Company's former subsidiary Celladon Europe B.V. (Celladon Europe) were consolidated with those of the Company through Celladon Europe's dissolution on December 30, 2014. All intercompany transactions and balances were eliminated in consolidation.

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 March 31, 2015 and December 31, 2014, 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 March 31, 2015	Maturity in Years	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Corporate debt securities	Less than 1 year	$ 20,571	$—	$ (1)	$20,570

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

Net Loss Per Share Attributable to Common Stockholders

Basic and diluted net loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period, without consideration for common stock equivalents. Potentially dilutive shares, which include 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 equity incentive plans, are considered to be common stock equivalents and are only included in the calculation of diluted net loss per share when their effect is dilutive.

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):

	Three Months Ended March 31,	
	2015	2014
Warrants for common stock	152,735	231,821
Common stock options and restricted stock units	3,400,097	2,060,890
	3,552,832	2,292,711

Recent Accounting Pronouncements

In April 2015, the FASB issued ASU No. 2015-03, Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs. The amendments in this ASU require that debt issuance costs related to a recognized debt liability be presented in the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. The amendments in this ASU are effective for financial statements issued for fiscal years beginning after December 15, 2015, and interim periods within those fiscal years. Early adoption of the amendments is permitted. The new guidance shall be applied on a retrospective basis, wherein the balance sheet of each individual period presented should be adjusted to reflect the period-specific effects of applying the new guidance. The Company is currently evaluating the impact of the adoption of this standard on its consolidated financial statements.

2. Balance Sheet Details

Prepaid expenses and other assets consist of the following (in thousands):

	March 31, 2015	December 31, 2014
Prepaid expenses	1,443	756
Commercial manufacturing costs (1)	—	1,751
Other receivables	158	628
Debt issue costs, current	72	—
	$ 1,673	$ 3,135

(1) The commercial manufacturing costs consisted mainly of design and engineering services for commercial drug manufacturing capabilities. The Company determined that it was probable that it would not complete the commercial manufacturing project in light of the negative CUPID 2 data. The Company therefore recorded the costs accumulated as of December 31, 2014 and activity in the first quarter of 2015 as a period expense in the consolidated financial statements in the three month period ending March 31, 2015.

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

	March 31, 2015	December 31, 2014
Office furniture and other equipment	$ 905	$ 881
Leasehold improvements	247	246
Construction in progress	81	—
Accumulated depreciation	(440)	(364)
	$ 793	$ 763

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

	March 31, 2015	December 31, 2014
Accounts payable	$ 2,790	$ 3,293
Accrued compensation	361	1,909
Accrued other	587	596
Current portion of deferred rent	8	5
	$ 3,746	$ 5,803

3. 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 are recorded at fair 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 March 31, 2015 and December 31, 2014, 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 liabilities that were measured or disclosed at fair value on a recurring basis, and were classified within the Level 3 designation, included the warrant liability and convertible notes prior to their conversion to equity upon the Company's initial public offering in February 2014. 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 March 31, 2015 and December 31, 2014 are all classified within Level 1. Below is a summary of other assets and liabilities measured at fair value (in thousands):

	As of March 31, 2015	Fair Value Measurements at Reporting Date Using		
		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	$ 20,570	$ —	$ 20,570	$ —

	As of December 31, 2014	Fair Value Measurements at Reporting Date Using		
		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	$ —

4. Commitments and Contingencies

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.

No expense was recorded related to sublicense fees under the agreement in each of the three month periods ended March 31, 2015 and March 31, 2014. Through March 31, 2015, 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 March 31, 2015, 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.

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 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 (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.

No expense was recorded related to license and annual maintenance fees under the agreement in each of the three month periods ended March 31, 2015 and March 31, 2014. Through March 31, 2015, 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.

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 $0.1 million for each of the three month periods ended March 31, 2015 and March 31, 2014.

Through March 31, 2015, 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. In March 2015, the Company entered into a short-term lease for approximately 7,000 square feet of office space in Seattle, Washington that expires in June 2016. 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 for the three months ended March 31, 2015 and March 31, 2014 was $0.1 million and $21,000, respectively. Future minimum payments under the long-term operating leases net of contractual sublease payments total $3.0 million.

5. Stockholders' Equity

Common Stock Warrants

The following table summarizes the fully exercisable warrants outstanding for the purchase of common stock as of March 31, 2015 and December 31, 2014:

March 31, 2015	December 31, 2014	Exercise Price	Expiration Date
152,735	206,340	$ 5.61	October 2018

Stock Options

Options granted under the Company's equity incentive plans generally expire no more than ten 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. The Company has also granted inducement stock options outside an equity incentive plan that are subject to the terms and conditions of the Company's 2013 Equity Incentive Plan.

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

The 2013 Equity Incentive Plan became effective in February 2014. Under the 2013 Equity Incentive Plan, the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units (RSUs), 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 Equity Incentive Plan provides for the grant of performance cash awards. The number of shares of common stock reserved for issuance under the 2013 Equity Incentive Plan will automatically increase on January 1 of each year 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 and RSU activity is as follows:

	Options and RSUs
Outstanding at December 31, 2014	2,408,634
Granted	1,302,750
Exercised	(296,287)
Cancelled	(15,000)
Outstanding at March 31, 2015	3,400,097

2013 Employee Stock Purchase Plan

The 2013 Equity Stock Purchase Plan (ESPP) became effective in January 2014. The number of shares of common stock reserved for issuance will automatically increase on January 1 of each calendar year 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).

Stock-Based Compensation Expense

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

	Three Months Ended March 31,	
	2015	2014
Research and development	$ 909	$343
General and administrative	1,194	176
	$2,103	$519

As of March 31, 2015 the unrecognized compensation cost related to outstanding employee options was $23.4 million and is expected to be recognized as expense over approximately 3.5 years.

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 up to $25.0 million was available for the Company to borrow in two tranches (the Loan).

The Company borrowed the first tranche of $10.0 million on August 1, 2014. The second tranche of up to $15.0 million was available to be drawn through June 30, 2015, but only if the Company provided the Lenders with notice that data from the Company's Phase 2b clinical trial for MYDICAR supported 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). In April 2015, the Company's senior management and board of directors determined that the Company did not achieve the Milestone (see Note 7). Accordingly, the Company cannot draw down the second tranche of $15.0 million. Pursuant to the terms of the Loan Agreement, the Company is required to begin repaying the Loan principal on August 1, 2015, in 30 equal monthly installments of principal and interest.

The Loan accrues interest 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 (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 in full 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. The Company is obligated to make 60 payments of approximately $600.

Contractual Payments and Carrying-Value Reconciliation

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 (in thousands):

	March 31, 2015	December 31, 2014
Total loan debt and capital lease obligations	$ 11,762	$ 11,762
Less: Debt discount	(1,381)	(1,659)
Total carrying value:	10,381	10,103
Less: Carrying value of current portion of long-term obligations	(1,671)	(1)
Carrying value of long-term obligations, less current portion	$ 8,710	$ 10,102

7. Subsequent Events

Results of MYDICAR Phase 2b Trial (CUPID 2)

On April 26, 2015, the Company announced that its Phase 2b CUPID 2 trial did not meet its primary and secondary endpoints. CUPID 2 is a randomized, double-blind, placebo-controlled, multinational trial evaluating a single, one-time, intracoronary infusion of the cardiovascular gene therapy agent MYDICAR(R) (AAV1/SERCA2a) versus placebo added to a maximal, optimized heart failure drug and device regimen. In the study, the primary endpoint comparison of MYDICAR to placebo resulted in a hazard ratio in the MYDICAR group of 0.93; 95% confidence interval (CI), 0.53 to 1.65; p=0.81, defined as heart failure-related hospitalizations or ambulatory treatment for worsening heart failure. The secondary endpoint comparison of MYDICAR to placebo, defined as all-cause death, need for a mechanical circulatory support device, or heart transplant, likewise failed to show a significant treatment effect. The efficacy endpoint analyses were performed on the (n=243) modified intent to treat population (mITT), which excludes clinical events that occurred in patients who did not receive MYDICAR or placebo, or which occurred prior to dosing. All other exploratory efficacy endpoints (improvement in New York Heart Association classification, 6 Minute Walk Test, Quality of Life, and NT-proBNP) were also inconsistent with a treatment effect. No safety issues were noted. More extensive review of the data is ongoing at this time.

Reduction in Force; Retention Program

In light of the CUPID 2 results, on April 26, 2015 the Company's board of directors approved an approximately 50% reduction of the Company's current full-time workforce of 34 employees in order to reduce operating expenses and conserve cash resources. The Company expects that a majority of employees included in this workforce reduction will be separated from the Company during the second quarter of 2015, with the remainder expected to be separated during the third quarter of 2015. The Company has also committed to retention payments payable to certain key employees if such employees remain with the Company until December 31, 2015 or are terminated by the Company without cause prior to such date. The Company estimates that it will incur aggregate cash charges of up to approximately $2.4 million associated with the workforce reduction and retention payments during 2015 including approximately $1.0 million in one-time severance payments, approximately $0.1 million in continuation of benefits, approximately $24,000 in outplacement service benefits and up to approximately $1.3 million by December 31, 2015 in connection with retention payments.

Termination of Supply Agreement

Effective April 29, 2015, the Company terminated the Development, Manufacturing and Supply Agreement by and between the Company and Novasep, Inc. (Novasep) dated March 20, 2015 (Novasep Agreement) pursuant to the Company's post CUPID 2 data termination right, after concluding that the recently un-blinded data from CUPID 2 was such that the Company does not require production of MYDICAR drug substance at Novasep's facility. The Company made payments to Novasep totaling €3.1 million through March 31, 2015 prior to terminating the Novasep Agreement and is scheduled to pay the remaining €1.7 million due in the second quarter of 2015.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following Management's Discussion and Analysis of Financial Condition and Results of Operations should be read in conjunction with our unaudited consolidated financial statements and related notes thereto included in this Quarterly Report on Form 10-Q and with our consolidated financial statements and the related notes thereto that are contained in our Annual Report on Form 10-K for the year ended December 31, 2014, or Annual Report, which has been filed with the Securities and Exchange Commission, or SEC. In addition to historical information, the following discussion and analysis includes forward-looking information that involves risks, uncertainties, and assumptions. Actual results and the timing of events could differ materially from those anticipated by these forward-looking statements as a result of many factors, including those discussed under "Risk Factors" elsewhere in this Form 10-Q and in our Annual Report.

Forward-Looking Statements

This Quarterly Report on Form 10-Q 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 include, but are not limited to, statements about:

- the continued development of MYDICAR for the treatment of heart failure for reduced ejection fraction, or HFrEF (also referred to as systolic heart failure), or any other indication;
- the success, cost and timing of our product development activities and clinical trials;
- our future operating expenses and other results of operations;
- our ability to obtain and maintain regulatory approval for MYDICAR, its 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 additional clinical trials that would be required to file a biologics license application, or BLA, and a Marketing Authorization Application, or MAA, for MYDICAR for the treatment HFrEF;
- the commercialization of our product candidates and any related companion diagnostics, if approved;
- our plans to research, develop and commercialize our product candidates and any related companion diagnostics;
- 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;
- potential future agreements with third parties in connection with the commercialization of MYDICAR, its 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 any related companion diagnostics;
- 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 Quarterly 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.

Overview

We are a clinical-stage biotechnology company with industry-leading expertise in the development of cardiovascular gene therapy. MYDICAR (AAV1/SERCA2a), our lead product candidate, uses gene therapy to target SERCA2a, which is an enzyme that becomes deficient in patients with heart failure, and has demonstrated evidence of therapeutic potential for the treatment of pulmonary hypertension (PAH) in animal models. 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. 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 currently in the long-term follow-up stage of a 250-patient randomized, double-blind, placebo-controlled multinational Phase 2b trial that was designed to evaluate MYDICAR in patients with HFrEF, which we refer to as the CUPID 2 trial. CUPID 2 evaluated a single, one-time, intracoronary infusion of the cardiovascular gene therapy agent MYDICAR versus placebo, in each case added to a maximal, optimized heart failure drug and device regimen. We completed enrollment of CUPID 2 in February 2014 and un-blinded the results from the active observation period in late April 2015.

On April 26, 2015, we announced that the CUPID 2 trial did not meet its primary and secondary endpoints. In the study, the primary endpoint comparison of MYDICAR to placebo, defined as heart failure-related hospitalizations or ambulatory treatment for worsening heart failure, resulted in a hazard ratio in the MYDICAR group of 0.93; 95% confidence interval (CI), 0.53 to 1.65; p=0.81. The secondary endpoint comparison of MYDICAR to placebo, defined as all-cause death, need for a mechanical circulatory support device, or heart transplant, likewise failed to show a significant treatment effect. The efficacy endpoint analyses were performed on the (n=243) modified intent to treat population (mITT), which excludes clinical events that occurred in patients who did not receive MYDICAR or placebo, or which occurred prior to dosing. All other exploratory efficacy endpoints (improvement in New York Heart Association classification, 6 Minute Walk Test, Quality of Life, and NT-proBNP) were also inconsistent with a treatment effect. No safety issues were noted.

We are in the process of conducting an extensive review of the CUPID 2 data. In the meantime, we have implemented cost-cutting measures, including a planned reduction in force and termination of certain contracts related to MYDICAR, and we are exploring strategic alternatives to maximize value for our stockholders. We have discontinued our plans for CELL-003, the previously planned follow-on multinational clinical trial of MYDICAR in HFrEF and are evaluating our other planned trials and development programs. We have also discontinued financial support and MYDICAR supply for the trial titled "Investigation of the Safety and Feasibility of AAV1/SERCA2a Gene Transfer in Patients with Heart Failure and a Left Ventricular Assist Device (LVAD)," which was partially funded by the British Heart Foundation and sponsored by Imperial College London. Following the CUPID 2 data and our decision to discontinue financial support, we were notified by the lead investigator of the LVAD trial that additional activities under the trial have been suspended, pending further review of additional information from the CUPID 2 data analysis.

On April 30, 2015, we announced a planned workforce reduction of approximately 50% of our current full-time workforce of 34 employees in order to reduce operating expenses and conserve cash resources. We expect that a majority of employees included in this workforce reduction will be separated during the second quarter of 2015, with the remainder expected to be separated during the third quarter of 2015. We have also committed to retention payments payable to certain key employees if such employees remain employed by us until December 31, 2015 or are terminated by us without cause prior to such date. We estimate that we will incur aggregate cash charges of up to approximately $2.4 million associated with the workforce reduction and retention plan payments during 2015 in connection with approximately $1.0 million in one-time severance payments, approximately $0.1 million in continuation of benefits, approximately $24,000 in outplacement service benefits and up to approximately $1.3 million by December 31, 2015 in connection with retention payments.

On March 20, 2015, we entered into a Development, Manufacturing and Supply Agreement with Novasep, Inc., or the Novasep Agreement, 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 future commercial manufacture of MYDICAR drug substance at the facilities of Novasep's affiliate in Europe. Effective April 29, 2015, we terminated the Novasep Agreement pursuant to our post CUPID 2 data termination right, after concluding that the recently

un-blinded CUPID 2 data was such that we do not require production of MYDICAR drug substance at Novasep's facility. We made payments to Novasep under the letter agreement and the Novasep Agreement totaling €3.1 million through March 31, 2015 and are scheduled to pay the remaining €1.7 million due in the second quarter of 2015.

Also in light of the CUPID 2 data, we do not intend to exercise the construction trigger under our Facility Construction and Commercial Supply Agreement with Lonza Biologics, Inc. dated October 31, 2014. Our failure to exercise the construction trigger or to pay a reservation extension fee to Lonza to secure an extension in advance of the near-term deadline will result in the automatic expiration of the agreement.

In July 2014, we entered into a Loan and Security Agreement with Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc., which we collectively refer to as Hercules, under which up to $25.0 million was available to us to borrow in two tranches. We borrowed the first tranche of $10.0 million on August 1, 2014. The second tranche of up to $15.0 million was available to be drawn through June 30, 2015, but only if we provided Hercules with notice that the CUPID 2 data supported 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 our senior management and board of directors. In April 2015, we determined that the CUPID 2 data did not support the continued development of MYDICAR for its Breakthrough Therapy designation to either a Phase 3 clinical trial or for registration for approval. Accordingly, we cannot draw down the remaining tranche of $15.0 million. Pursuant to the terms of the agreement, we are required to begin repaying the principal amount of the loan on August 1, 2015, in 30 equal monthly installments of principal and interest.

To date, we have devoted substantially all of our resources to research and development efforts relating to our product candidates, including conducting clinical trials and developing manufacturing capabilities, in-licensing related intellectual property, providing general and administrative support for these operations and protecting our intellectual property. We do not have any products approved for sale and have not generated any revenue from product sales or other sources. From our inception through March 31, 2015, we have funded our operations primarily through the sales of equity and debt securities totaling approximately $219.9 million.

We have incurred net losses in each year since our inception. As of March 31, 2015, we had an accumulated deficit of approximately $163.2 million. Substantially all of our net losses, including those incurred during the periods presented in this report, have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We are currently evaluating our research and development programs and plan to assess the viability of continuing to pursue one or more of these programs. We cannot predict our future cash needs until we complete this analysis.

If we decide to continue the development of one or more of our programs, we expect to continue to incur significant expenses and increasing losses for at least the next several years. We would need to raise additional capital in order to conduct additional clinical trials of MYDICAR that will be required for marketing approval and to further the development of its companion diagnostic, our small molecule program, or any 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 any related 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 deploy the capital necessary to refocus or expand our operations or otherwise capitalize on our business opportunities, as desired, any of which could materially adversely affect our business, financial condition and results of operations and could even require us to cease operations entirely.

Financial Operations 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 March 31, 2015, we have incurred approximately $126.2 million in research and development expenses, of which we estimate $119.9 million relates to our development of MYDICAR. If we decide to continue the development of MYDICAR for the treatment of HFrEF and the development of its companion diagnostic, and/or further advance the development of our other programs and/or MYDICAR for additional indications, we would need to increase our research and development expenses over time. 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 any related companion diagnostics 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 any related companion diagnostics 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 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; 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. In light of the negative CUPID 2 data, if we decide to continue the development of MYDICAR for HFrEF, we will be required to expend significant additional financial resources and time with respect to the development of MYDICAR and its companion diagnostic.

MYDICAR-HFrEF

The majority of our research and development resources until recently have been focused on our 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 and we are currently evaluating strategic alternatives in light of the negative CUPID 2 trial results. We have incurred, and if we continue the development of MYDICAR, expect to continue to incur, significant expense in connection with these efforts, including expenses related to:

- conduct of potential future clinical trials;
- future scale-up, validation and automation activities related to the companion diagnostic for MYDICAR; and
- the development of manufacturing capabilities for the production of MYDICAR.

MYDICAR-PAH

Our research and development expenses for MYDICAR for PAH relate primarily to the preclinical testing in porcine models of PAH.

Stem Cell Factor Program

Our research and development expenses for our stem cell factor program relate primarily to the preclinical testing of the membrane-bound form of the Stem Cell Factor gene, or mSCF in myocardial infarction porcine models.

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. We have suspended initiating further activities on this program pending our review of strategic alternatives.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for employees in executive, finance, legal 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, expenses associated with pre-commercial planning initiatives, the cost of various consultants, occupancy costs and information systems costs. We expect our general and administrative expenses to decrease compared to prior periods for the foreseeable future due to a planned reduction in workforce and recently suspended activities related to pre-commercial planning as we evaluate strategic alternatives in light of the negative CUPID 2 data.

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. We expect our interest expense to decrease starting in the third quarter of 2015 as we begin repaying our loan principal in August 2015. Other income consists primarily of interest income earned on our cash, cash equivalents and investments. We expect our interest income to decrease as we reduce our investment balance to fund current operations.

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 further described in Note 1 to our consolidated financial statements appearing elsewhere in this Form 10-Q, we believe that the following accounting policies related to clinical trial expenses, valuation of stock-based compensation and valuation of our convertible debt and warrant liability 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 March 31, 2015, 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

Stock-based compensation expense represents the grant date fair value of employee equity grants recognized over the requisite service period of the awards (usually the vesting period) on a straight-line basis, net of estimated forfeitures. For awards 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 awards granted to non-employees using the fair-value approach. These awards are subject to periodic revaluation over their vesting terms.

We estimate the fair value of our stock options granted to employees and non-employees 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.

Recent Accounting Pronouncements

See Item 1 of Part I, "Notes to Consolidated Financial Statements — Note 1 — Basis of Presentation, Organization and Summary of Significant Accounting Policies" of this Quarterly Report on Form 10-Q.

Results of Operations

Comparison of the Three Months Ended March 31, 2015 and 2014

The following table summarizes our results of operations for the three months ended March 31, 2015 and 2014 (in thousands):

	Three Months Ended March 31,		Increase / (Decrease)
	2015	2014	
Research and development	$11,518	$5,218	$ 6,300
General and administrative	4,779	1,706	3,073
Total other (expense) income	(449)	(238)	(211)

Research and Development Expenses. Research and development expenses were $11.5 million and $5.2 million for the three months ended March 31, 2015 and 2014, respectively. The increase of approximately $6.3 million was due primarily to an increase of $5.2 million in expenses incurred during the first quarter of 2015 associated with drug substance manufacturing scale-up, $0.6 million in stock-based compensation due to additional grants, $0.4 million in compensation related to an increase in headcount, $0.4 million in clinical consulting, $0.4 million in preclinical costs and $0.2 million in rent and various other costs offset by a decrease of $0.9 million in clinical costs due to the completion of enrollment in our CUPID 2 trial in the first quarter of 2014. We anticipate a reduction in our research and development expenses for the foreseeable future due to a planned reduction in workforce and certain recently suspended MYDICAR related activities while we reevaluate our programs and strategic alternatives in light of the negative CUPID 2 data.

General and Administrative Expenses. General and administrative expenses were $4.8 million and $1.7 million for the three months ended March 31, 2015 and 2014, respectively. The increase of approximately $3.1 million was due to an increase of $1.1 million in pre-commercial planning efforts, $1.0 million in stock-based compensation due to additional grants, $0.5 million in

compensation related to an increase in headcount and $0.5 million in legal, insurance and various other public company related costs. We expect our general and administrative expenses to decrease in the foreseeable future due to a planned reduction in workforce and the recently suspended activities related to pre-commercial planning efforts as we evaluate our strategic alternatives in light of the negative CUPID 2 data.

Other Expense. Other expense was $0.4 million and $0.2 million for the three months ended March 31, 2015 and 2014, respectively. Other expense for the three months ended March 31, 2015 consisted primarily of $0.5 million of expense related to the accretion of debt discount and interest charges on our term loan offset by $40,000 in interest and other income. Other expense for the three months ended March 31, 2014 consisted primarily of the change in fair value of the warrant liability prior to its reclassification to stockholders' equity in February 2014 in connection with the closing of our initial public offering.

Liquidity and Capital Resources

We have incurred net losses each year since our inception and as of March 31, 2015, we had an accumulated deficit of approximately $163.2 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 decrease for the foreseeable future due to a planned reduction in workforce and certain recently suspended MYDICAR activities and pre-commercial planning efforts while we reevaluate our programs and strategic alternatives in light of the negative CUPID 2 data. We expect that we may 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. As a result of the negative CUPID 2 results, we cannot draw down the second tranche of our loan facility with Hercules in the amount of $15.0 million and we expect to begin repaying the $10.0 million principal borrowed on August 1, 2015, in 30 equal monthly installments of principal and interest.

Since our inception through March 31, 2015, we have funded our operations primarily through the sale of our equity and debt securities. As of March 31, 2015, we had cash, cash equivalents and investments of approximately $70.6 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

The following table summarizes our cash flows for the periods indicated (in thousands):

	Three Months Ended March 31,	
	2015	2014
Net cash provided by (used in):		
Operating activities	$(14,765)	$ (6,547)
Investing activities	49,712	6,488
Financing activities	636	46,033
Net increase (decrease) in cash and cash equivalents	$ 35,583	$45,974

Operating activities. Net cash used in operating activities of $14.8 million during the three months ended March 31, 2015 was primarily a result of our net loss of $16.7 million. The primary difference between our net loss and our cash used in operating activities was $2.1 million of stock-based compensation, $0.3 million of non-cash interest related to the accretion of debt discount on our term loan with Hercules, $0.1 million amortization of premiums paid on investment securities, $0.1 million of depreciation expense and $(0.6) million of changes in our operating assets and liabilities.

Net cash used in operating activities of $6.5 million during the three months ended March 31, 2014, was primarily a result of our net loss of $7.1 million. The primary difference between our net loss and our cash used in operating activities was $(0.2) million of changes in our operating assets and liabilities, $0.5 million of stock-based compensation, $0.2 million related to the change in fair value of our outstanding warrant liability and $0.1 million of non-cash interest related to the amortization of debt discount on our convertible debt.

Investing Activities. Net cash provided by investing activities of $49.7 million and $6.5 million during the three months ended March 31, 2015 and 2014, respectively, was primarily a result of the net maturities of investments used to fund our operating activities. In 2015 and 2014, amounts of $0.1 and $0.2 million, respectively, were also used to purchase property and equipment.

Financing Activities. Net cash provided by financing activities during the three months ended March 31, 2015 consisted of $0.6 million in proceeds received upon the exercise of employee stock options. Net cash provided by financing activities of $46.0 million during the three months ended March 31, 2014 consisted of $50.6 million in proceeds received and $4.6 million in costs paid in connection with our initial public offering.

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 expect that our expenses will decrease for the foreseeable future due to a planned reduction in workforce and certain recently suspended MYDICAR programs and pre-commercial planning activities while we reevaluate our programs and strategic alternatives in light of the negative CUPID 2 data. We anticipate that we may 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 will enable us to fund our operations for at least the next 12 months. We are currently evaluating various strategic alternatives for the use of our existing cash, cash equivalents and short-term investments. We have based our planning 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 product candidates, we are unable to estimate the amounts of capital outlays and operating expenditures that would be necessary to complete the development of our product candidates.

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

- the outcome of our evaluation of strategic alternatives in light of the negative CUPID 2 data;
- the potential continued clinical development of our product candidates;
- 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 refocus or 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 retain or hire additional management and scientific, medical and sales personnel; and
- the effect of competing technological and market developments.

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 March 31, 2015 (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	$2,462	$ 9,295	$ 5	$ —
Interest commitment on long-term obligations (1)	1,412	784	628	—	—
Operating lease obligations	3,168	647	1,007	846	668
Total	$16,342	$3,893	$10,930	$851	$ 668

(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. We will begin repaying the $10 million principal currently outstanding in 30 equal payments of principal and interest starting in August 2015. 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.

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 SEC.

Item 3. 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 March 31, 2015, 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 March 31, 2015 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 March 31, 2015, we had €1.7 million in contractual payment obligations denominated in euros pursuant to our Development, Manufacturing and Supply Agreement with Novasep. We have ongoing clinical trial agreements denominated in euros. 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 three month period ended March 31, 2015. A 10% change in the euro-to-dollar exchange rate on March 31, 2015 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 4. 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 Securities Exchange Act of 1934, as amended, or 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 March 31, 2015, the end of the period covered by this report.

Changes in Internal Control over Financial Reporting.

There were no changes in our internal control over financial reporting during the quarter ended March 31, 2015 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. 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 1A. Risk Factors

You should carefully consider the following risk factors, as well as the other information in this report, before deciding whether to purchase, hold or sell shares of our common stock. 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 factors described when evaluating our business. The risk factors set forth below that are marked with an asterisk (*) did not appear as separate risk factors in Item 1A of our Annual Report or contain changes to the similarly titled risk factors included in Item 1A of our Annual Report. If any of the following risks actually occurs, our business, financial condition, results of operations and future growth prospects would likely be materially and adversely affected. In these circumstances, the market price of our common stock would likely decline.

Risks Related to our Business

Our business to date has been almost entirely dependent on the success of MYDICAR, which recently failed to show a treatment effect in the Phase 2b clinical trial known as CUPID 2. As we analyze the data from CUPID 2 and conduct a review of strategic alternatives, we may decide not to continue development of MYDICAR or our other programs.*

On April 26, 2015, we announced that the CUPID 2 trial did not meet its primary and secondary endpoints. CUPID 2 is a 250-patient randomized, double-blind, placebo-controlled multinational Phase 2b trial that was designed to evaluate MYDICAR in patients with HFrEF. We had previously devoted substantially all of our research, development and clinical efforts and financial resources toward the development of MYDICAR. In an effort to conserve our cash resources and reduce operating expenses, we have approved a workforce reduction of approximately one-half of our workforce and the termination of our development, manufacturing and supply agreement with Novasep. We are currently analyzing the CUPID 2 data and are assessing whether to continue the development of MYDICAR for HFrEF and/or other potential indications, as well as conducting a review of our other programs and strategic alternatives. We cannot predict the outcome of this review or whether we will decide to continue with drug development.

The other programs in our pipeline are in early stages of development and our efforts to develop and commercialize any product candidates that could result from these programs are subject to a high risk of failure. If we fail to successfully develop product candidates, our ability to generate revenues will be substantially impaired.*

The process of successfully developing product candidates for the treatment of human diseases is very time-consuming, expensive and unpredictable and there is a high rate of attrition for product candidates in preclinical and clinical trials. Until recently, our business strategy depended upon the successful clinical development of MYDICAR and the subsequent development of additional pipeline product candidates to complement our initial focus on MYDICAR. Our remaining programs are in much earlier stages of development than MYDICAR, and would require substantial additional financial resources, as well as research, development and clinical activities, to pursue the development of these product candidates, and we may never develop an approvable product.

As a result of the CUPID 2 data and the reduction in our workforce that we announced in April 2015, we may not be successful in retaining key employees. If we are unable to retain our management, scientific staff and scientific advisors, our business will be seriously jeopardized.*

On April 30, 2015, we announced a planned workforce reduction of approximately one-half of our workforce. Our cash conservation activities may yield unintended consequences, such as attrition beyond our planned reduction in workforce and reduced employee morale which may cause our remaining employees to seek alternate employment. Competition among biotechnology

companies for qualified employees is intense, and the ability to retain our key employees is critical to our ability to effectively manage our resources following the CUPID 2 data and for defining a path forward for the company. Although we have implemented a retention program for certain key employees who will each receive a retention payment equal to 50% of their salary if they remain employed by us until December 31, 2015 (or are terminated prior to that date other than for cause), our retention plan may not be successful in incentivizing these employees to stay employed with us. Additional attrition could have a material adverse effect on our business. In addition, as a result of the reduction in our workforce, we face an increased risk of employment litigation.

Furthermore, while we have entered into employment 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. The failure of CUPID 2 will likely make it more challenging to retain qualified personnel, and difficult to recruit personnel in the future, if necessary. The inability to recruit or loss of the services of any executive, key employee, consultant or advisor may impede our ability to identify and execute on a strategic path forward.

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 March 31, 2015, we had an accumulated deficit of approximately $163.2 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 operating losses for the foreseeable future if and as we:

- continue the development of MYDICAR for HFrEF or other indications;
- further validate and develop the manufacturing process for MYDICAR and its 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 any required companion diagnostics 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 originally anticipated. 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, in August 2014 we borrowed $10.0 million from Hercules. 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. 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 the outstanding amount under the loan agreement may become due and payable immediately, but we may not have access to such funds on reasonable terms or at all, which could harm our liquidity, business, financial condition, operating results and prospects. In addition, although the CUPID 2 data did not, in and of itself result in a default under the loan agreement, there is no guarantee that future negative business conditions will not flow from the CUPID 2 data which could result in an event of default.

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 may need to raise substantial additional funding to the extent we continue our product development efforts, 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 or other operations.*

Our operations have consumed substantial amounts of cash since inception. As of March 31, 2015, our cash, cash equivalents and investments were approximately $70.6 million. Our research and development expenses were $11.5 million and $5.2 million for the three months ended March 31, 2015 and 2014, respectively. 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, we are currently conducting a review of strategic alternatives and our operating plan may be impacted by 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 continue the development of our product candidates and companion diagnostic, as well as to further develop MYDICAR, if we decide to do so.

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. 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 any required companion diagnostic(s), 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 deploy the capital necessary to refocus or expand our operations or otherwise capitalize on our business opportunities, as desired, any of which could materially adversely affect our business, financial condition and results of operations and could even require us to cease operations entirely.

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

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 further and existing stockholders may not agree with our financing plans or the terms of such financings. Given the negative results of our CUPID 2 trial, we believe our ability to issue equity securities or obtain debt financing in the future on favorable terms, or at all, has been substantially impaired, particularly if the intended use of proceeds would be for the continued development of MYDICAR.

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 in order to continue the development of MYDICAR and our other product candidates. 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 may not be able to continue the development of, successfully obtain regulatory or marketing approval for, or successfully commercialize, MYDICAR.*

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. The setback caused by the unfavorable CUPID 2 outcome has resulted in a development delay of at least a few years. If we decide to continue the development of MYDICAR for HFrEF, 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 European Economic Area (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. If we elect to continue with our MYDICAR product development efforts, 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.

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. In addition, our negative CUPID2 data may adversely affect the attitude of regulatory authorities toward continued development of MYDICAR.*

We have primarily concentrated our research and development efforts on our lead product candidate, MYDICAR, for the treatment of HFrEF. 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, if we decide to continue the development of MYDICAR and ultimately pursue regulatory approval, the FDA will require 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. Furthermore, the negative results from the CUPID 2 trial could result in more stringent requirements being imposed by the regulatory bodies and advisory groups, should we decide to continue the development of MYDICAR.

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 prevent commercialization of MYDICAR. Devices used in the administration of MYDICAR may also require labeling changes and result in delays or complications for the commercialization of MYDICAR.*

A key element of our MYDICAR 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 to help us better identify those patients who may benefit from treatment with MYDICAR. If we decide to continue the development of MYDICAR, 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. If we decide to continue the development of MYDICAR, we expect that we would 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. In connection with the potential future commercialization of MYDICAR, it is possible that MYDICAR could 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 and complicate 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, especially in light of our CUPID 2 data.*

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. In light of the failed CUPID 2 efficacy outcome, it is possible that patients will be less willing to participate in the on-going or any future potential trial of MYDICAR. 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 clinical trials of MYDICAR 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 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, we may need to delay, limit or terminate ongoing or future 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. While 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 provide ongoing benefits to us in light of our negative CUPID 2 data, and may be formally withdrawn by the FDA. 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.

Our CUPID 2 results cast serious doubt about the probative value of our success in earlier, smaller clinical trials.*

Trial designs and results from previous trials are not necessarily predictive of our future clinical trial designs or results. For example, although the results of our Phase 2a clinical trial of MYDICAR for the treatment of for HFrEF (CUPID 1) were positive, our Phase 2b clinical trial of MYDICAR (CUPID 2) failed to meet its primary or secondary endpoints. 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, including us. 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.

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. If we decide to initiate dosing in 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 Biologic Licence Application, or 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, coupled with our CUPID 2 results, 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 addition, the results of CUPID 2 may have adversely affected public opinion of MYDICAR. 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 a Marketing Authorization Application, or 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 a Special Protocol Assessment, or 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 a New Drug Application, or 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.

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 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 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 one supplier, Lonza, for the manufacturing of MYDICAR drug substance for clinical testing purposes. We expect to rely upon Lonza, and/or other third parties to produce materials required for the commercial production of our product candidates and companion diagnostic if we continue the further development of one or more of our programs and 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 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. 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 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, 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.

If our 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 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.

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.

If we are unable to build our own sales force or negotiate a strategic partnership for the commercialization of MYDICAR or its 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 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. Separately, as we continue to analyze the CUPID 2 data, we could identify further limitations on the patient population potentially eligible for MYDICAR treatment. If the potential eligible patient population is lower than we previously anticipated, or if considerably more than 60% of potential patients exhibit NAbs, our business, financial condition and results of operations could be significantly and negatively impacted.

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, if approved, and change the standard of care for heart failure patients include Renova Therapeutics, NanoCor Therapeutics, Juventas Therapeutics, VentriNova, uniQure N.V. in partnership with Bristol Myers Squibb 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, its 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

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. Following the announcement of our CUPID 2 data in late April 2015, our stock price decreased substantially, which may invite securities class action litigation against us. 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 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 identifying or discovering additional product candidates.*

Our research programs, if continued, 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 our 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. Also, the setback to our MYDICAR development program that resulted from the failure of CUPID 2 could also reduce the benefits of patents covering MYDICAR given the increased timelines to potential commercialization. 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 our 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. Since our initial public offering in January 2014 at a price of $8.00 per share, the sale price of stock as reported on The NASDAQ Global Market has ranged from $2.32 to $28.25, through May 8, 2015. 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;
- announcements of significant changes in our business or operations, including the decision not to pursue one or more of our drug development programs;
- 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, manufacture 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, future development programs or general and administrative expenses (including as the result of a significant decrease or increase in employee headcount);
- 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.

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 2. Unregistered Sales of Equity Securities and Use of Proceeds

Recent Sales of Unregistered Securities

On March 11, 2015, we issued an aggregate of 8,581 shares of our common stock to Venrock Partners, L.P., Venrock Associates IV, L.P., and Venrock Entrepreneurs Fund IV, L.P. pursuant to the net exercise provisions of warrants to purchase 10,946 shares of our common stock at an exercise price of $5.61. On March 13, 2015, we issued 33,692 shares of our common stock to Pfizer Inc. pursuant to the net exercise provisions of a warrant to purchase 42,659 shares of our common stock at an exercise price of $5.61 per share. No cash was paid to us for the foregoing issuances of our common stock. 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.

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. As of March 31, 2015, we have used approximately $26.5 million of the net proceeds from our initial public offering. We are evaluating various strategic alternatives to maximize shareholder value and our management will have broad discretion in the application of the remaining proceeds.

Item 3. Defaults Upon Senior Securities

Not applicable.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

On May 13, 2015, the Compensation Committee of our Board of Directors approved expanding our previously announced retention program to include seven additional key employees, including Elizabeth E. Reed, our Vice President and General Counsel. Pursuant to the retention program, each key employee will be eligible to receive a lump sum retention payment equal to 50% of the employee's base salary if such employee remains employed by us until December 31, 2015, or if such employee is terminated by us without cause prior to such date. Ms. Reed's current base salary is $280,500, and accordingly she will be eligible to receive a lump sum retention payment equal to $140,250 pursuant to the retention program. We estimate that we will incur aggregate cash charges of up to approximately $0.7 million by December 31, 2015 in connection with this expansion to the retention program.

Item 6. Exhibits

A list of the exhibits filed as part of this Quarterly Report on Form 10-Q is set forth on the Exhibit Index, which is incorporated herein by reference.

SIGNATURES

Pursuant to the requirements 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

Dated: May 14, 2015

/s/ Krisztina M. Zsebo, Ph.D.
Krisztina M. Zsebo, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

Dated: May 14, 2015

/s/ Paul B. Cleveland
Paul B. Cleveland
President and Chief Financial Officer
(Principal Financial Officer)

EXHIBIT INDEX

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*	Development, Manufacturing and Supply Agreement, dated March 20, 2015, by and between the Registrant and Novasep, Inc.
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

* Confidential treatment has been requested with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.

Exhibit 10.1

*****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) […***…].

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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:

[…***…]

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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.

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.

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 […***…]

[…***…].

(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

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 twenty six million one hundred sixty thousand Euro (26 160 000 €). 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) Development Services Fees:

(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 three million Euro (3 000 000 €), 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.

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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).

***Confidential Treatment Requested

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

***Confidential Treatment Requested

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 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 Quarterly Report on Form 10-Q 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: May 14, 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 Quarterly Report on Form 10-Q 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: May 14, 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 Quarterly Report on Form 10-Q 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: May 14, 2015

/s/ Krisztina M. Zsebo, Ph.D.
Krisztina M. Zsebo, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350 and is not being filed as part of the Report or as a separate disclosure document.

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED 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 Quarterly Report on Form 10-Q 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: May 14, 2015

/s/ Paul B. Cleveland
Paul B. Cleveland
President and Chief Financial Officer
(Principal Financial Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350 and is not being filed as part of the Report or as a separate disclosure document.