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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 September 30, 2014

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 October 31, 2014 was 23,304,631.

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CELLADON CORPORATION

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FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2014

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

Celladon Corporation

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

	September 30, 2014 (unaudited)	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 14,367	$ 7,903
Short-term investments	80,755	10,467
Prepaid expenses and other assets	891	180
Total current assets	96,013	18,550
Property and equipment, net	616	308
Other assets	280	2,296
Total assets	$ 96,909	$ 21,154
Liabilities, preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable and accrued expenses	$ 3,959	$ 2,908
Accrued clinical expenses	867	1,478
Accrued interest	86	14
Convertible notes, net of discount	—	1,044
Warrant liability	—	1,116
Total current liabilities	4,912	6,560
Term loan, net of discount	9,911	—
Non-current liabilities	111	37
Commitments and contingencies (Note 5)		
Series A-1 redeemable convertible preferred stock, $0.0001 par value:		
Authorized shares — none and 135,826,497 at September 30, 2014 and December 31, 2013, respectively; issued and outstanding shares — none and 127,140,530 at September 30, 2014 and December 31, 2013, respectively; liquidation preference — none and $114,172 at September 30, 2014 and December 31, 2013, respectively	—	60,098
Convertible preferred stock, $0.0001 par value:		
Authorized shares — none and 12,138,080 at September 30, 2014 and December 31, 2013, respectively; issued and outstanding shares — none and 12,138,080 at September 30, 2014 and December 31, 2013, respectively; liquidation preference — none and $5,450 at September 30, 2014 and December 31, 2013, respectively	—	5,450
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value; authorized shares — 10,000,000 and none at September 30, 2014 and December 31, 2013, respectively; no shares issued and outstanding	—	—
Common stock, $0.001 par value; authorized shares — 200,000,000 and 180,000,000 at September 30, 2014 and December 31, 2013, respectively; issued and outstanding — 23,297,567 and 884,179 at September 30, 2014 and December 31, 2013, respectively	23	—
Additional paid-in capital	217,058	61,593
Accumulated other comprehensive (loss) income	(8)	2
Accumulated deficit	(135,098)	(112,586)
Total stockholders' equity (deficit)	81,975	(50,991)
Total liabilities, preferred stock and stockholders' equity (deficit)	$ 96,909	$ 21,154

See accompanying notes.

Celladon Corporation

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Operating expenses:				
Research and development	$ 5,316	$ 4,571	$ 15,515	$ 11,707
General and administrative	2,815	952	6,545	2,280
Total operating expenses	8,131	5,523	22,060	13,987
Loss from operations	(8,131)	(5,523)	(22,060)	(13,987)
Other income (expense):				
Interest income	36	14	65	58
Interest expense	(271)	—	(330)	—
Other income (expense)	8	56	(4)	17
Change in fair value of warrant liability	—	—	(183)	—
Consolidated net loss	(8,358)	(5,453)	(22,512)	(13,912)
Net loss attributable to non-controlling interest	—	—	—	96
Net loss attributable to Celladon Corporation	(8,358)	(5,453)	(22,512)	(13,816)
Change in fair value of non-controlling interest	—	—	—	(3,105)
Net loss attributable to common stockholders	$ (8,358)	$ (5,453)	$ (22,512)	$ (16,921)
Other comprehensive income (loss):				
Unrealized gain (loss) on investments	(26)	5	(10)	(2)
Comprehensive loss	$ (8,384)	$ (5,448)	$ (22,522)	$ (13,914)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.40)	$ (6.17)	$ (1.32)	$ (19.14)
Weighted-average shares outstanding, basic and diluted	20,752,895	884,179	16,999,766	884,179

See accompanying notes.

Celladon Corporation

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

	Nine Months Ended September 30,	
	2014	2013
Cash flows from operating activities		
Consolidated net loss	$(22,512)	$(13,912)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation	110	49
Stock-based compensation	2,178	1,078
Noncash interest expense	190	—
Amortization of investment premium (discount)	163	230
Change in fair value of warrant liability	183	—
Other items, net	85	19
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(689)	46
Accounts payable and accrued expenses	910	1,113
Other liabilities	8	—
Net cash used in operating activities	(19,374)	(11,377)
Cash flows from investing activities		
Purchases of investment securities	(88,661)	(16,612)
Proceeds from maturities of investment securities	18,200	24,792
Purchases of property and equipment	(549)	(12)
Net cash (used in) provided by investing activities	(71,010)	8,168
Cash flows from financing activities		
Proceeds from issuance of common stock	94,578	—
Costs paid in connection with common stock offerings	(7,396)	(677)
Proceeds from borrowing under term loan	10,000	—
Costs paid in connection with term loan	(334)	—
Net cash provided by (used in) financing activities	96,848	(677)
Net increase (decrease) in cash and cash equivalents	6,464	(3,886)
Cash and cash equivalents, beginning of period	7,903	13,841
Cash and cash equivalents, end of period	$ 14,367	$ 9,955

See accompanying notes.

Celladon Corporation

Notes to Consolidated Financial Statements

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, 2013 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. The Company applies its leadership position in the field of gene therapy and calcium dysregulation to develop novel therapies for diseases with tremendous unmet medical needs and characterized by an underlying SERCA enzyme deficiency.

As of September 30, 2014, the Company has devoted substantially all of its efforts to product development, raising capital and building infrastructure and has not generated revenues from its planned principal operations.

Public Offerings

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

In August 2014, the Company completed an underwritten public offering of 4,600,000 shares of common stock at an offering price of $9.50 per share, which included the exercise by the underwriters of their option to purchase 600,000 additional shares of common stock. The Company received net proceeds of $40.7 million after deducting underwriting discounts and commission and offering expenses payable by the Company, including $0.3 million in offering costs paid by the Company after September 30, 2014. See Note 6 for additional information.

Principles of Consolidation

In April 2012, Celladon formed a subsidiary, Celladon Europe B.V. (Celladon Europe). From its inception to June 6, 2013, the subsidiary was approximately 90% owned by Celladon and subsequent to June 6, 2013 the subsidiary has been wholly owned by Celladon. The financial statements of Celladon Europe are consolidated with those of the Company. All intercompany transactions and balances are eliminated in consolidation. The U.S. dollar is the functional currency of Celladon Europe. The Company remeasures Celladon Europe's assets and liabilities related to monetary assets and liabilities to the U.S. dollar and records the net gains or losses resulting from remeasurement in other income (expense) in the consolidated statements of operations and comprehensive loss. Since the formation of Celladon Europe, the Company did not record any material gains or losses from remeasurement.

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 September 30, 2014 and December 31, 2013, none of the investment securities have been in an unrealized loss position for more than 12 months. Premiums and discounts are amortized or accreted over the life of the related security as an adjustment to yield using the straight-line method and are included in interest income. Interest income is recognized when earned.

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

As of September 30, 2014	Maturity in Years	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Corporate debt securities	Less than 1 year	$ 80,763	$—	$ (8)	$80,755

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

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):

	Nine Months Ended September 30,	
	2014	2013
Redeemable convertible preferred stock	—	10,179,372
Convertible preferred stock	—	971,820
Warrants for common stock	206,340	702
Common stock options	2,527,067	1,359,090
	2,733,407	12,510,984

Recent Accounting Pronouncements

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

In June 2014, the FASB issued Accounting Standards Update (ASU) No. 2014-10, *Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*. ASU 2014-10 a) eliminates the requirement for development stage entities to present inception-to-date information in the statements of income, cash flows and shareholder equity, b) amends Topic 275 to clarify that the risk and uncertainty disclosure requirements apply to entities that have not commenced principal operations, c) eliminates the exception related to the sufficiency of equity at risk for development stage entities from the guidance on variable interest entities in paragraph 810-10-15-16 to increase consistency in application of consolidated guidance across all entities and d) removes the definition of *development stage entities* from the Master Glossary of the Accounting Standards Codification. The amendments in this Update are to be applied retrospectively except for the clarification to Topic 275, which shall be applied prospectively. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2014, with an option for early adoption. The Company adopted this guidance prior to filing this Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2014. The adoption of ASU 2014-10 impacted disclosure only and did not have any impact on financial position or results of operations.

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

2. Celladon Europe B.V.

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

The redeemable non-controlling interest was initially valued using the fair value of the Series A-1 preferred stock. At each reporting period, the Company adjusted the carrying value of the redeemable non-controlling interest by the net loss attributable to the redeemable non-controlling interest. Any difference between the fair value and the adjusted carrying value of the redeemable non-controlling interest was recorded as an adjustment to additional paid-in capital and presented as a component of net loss attributable to common stockholders in the accompanying consolidated statements of operations and comprehensive loss.

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

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

During the nine months ended September 30, 2013, the Company adjusted the loss attributable to common stockholders as a result of increases in the fair value of the redeemable non-controlling interest of approximately $3.1 million. The increase in fair value increased the loss attributable to common stockholders.

In May 2014, the Company completed the transfer of the open clinical site contracts from Celladon Europe to Celladon. As of September 30, 2014, there were no liabilities recognized as a result of consolidating Celladon Europe. As of December 31, 2013, the $0.8 million of liabilities recognized as a result of consolidating Celladon Europe did not represent additional claims on the Company's general assets; rather, they represented claims against the specific assets of Celladon Europe. As of September 30, 2014 and December 31, 2013, the $0.1 million and $0.6 million, respectively, of assets recognized as a result of consolidating Celladon Europe did not represent additional assets that could be used to satisfy claims against the Company's general assets. The assets of Celladon Europe represent the only significant assets of the Company not located in the United States.

3. Balance Sheet Details

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

	September 30, 2014	December 31, 2013
Office furniture and other equipment	$ 937	$ 555
Accumulated depreciation	(321)	(247)
	$ 616	$ 308

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

	September 30, 2014	December 31, 2013
Accounts payable	$ 2,198	$ 1,397
Current portion of deferred rent	—	8
Accrued compensation	1,179	664
Accrued other	582	839
	$ 3,959	$ 2,908

4. Fair Value Measurements

The Company's financial instruments primarily consist of cash and cash equivalents, investment securities, accounts payable and accrued liabilities. The carrying value of these financial instruments generally approximates fair value due to their short-term nature. Investment securities 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 September 30, 2014 and December 31, 2013, cash and cash equivalents consist primarily of bank deposits with third-party financial institutions and highly liquid money market securities with original maturities at date of purchase of 90 days or less and are stated at cost which approximate fair value and are classified within the Level 1 designation discussed above. Marketable securities are recorded at fair value, defined as the exit price in the principal market in which the Company would transact, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. Level 2 securities are valued using quoted market prices for similar instruments, non-binding market prices that are corroborated by observable market data, or discounted cash flow techniques and include the Company's investments in corporate debt securities and commercial paper. Financial 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 September 30, 2014 and December 31, 2013 are all classified within Level 1. Below is a summary of other assets and liabilities measured at fair value (in thousands):

	As of September 30, 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	$ 80,755	$ —	$ 80,755	$ —

	As of December 31, 2013	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	$ 10,467	$ —	$ 10,467	$ —
Liabilities:				
Convertible notes	$ 1,044	$ —	$ —	$ 1,044
Warrant Liability	1,116	—	—	1,116
	$ 2,160	$ —	$ —	$ 2,160

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

The fair value per share of the Company's underlying Series A-1 preferred stock was used to determine the fair value of the warrant liability. As of December 31, 2013, the fair value of the Series A-1 preferred stock was $0.64 and was derived from the price at which shares were sold in the Company's initial public offering.

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

	December 31, 2013
Risk-free interest rate	1.58%
Expected volatility	82%
Expected term (in years)	4.8
Expected dividend yield	0.0%

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

	Convertible Notes	Warrant Liability
Balance at December 31, 2013	$ 1,044	$ 1,116
Changes in fair value	53	183
Conversion to equity upon initial public offering	(1,097)	(1,299)
Balance at September 30, 2014	$ —	$ —

5. 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.

The Company recorded research and development expense related to sublicense fees under the agreement of $0.3 million in each of the nine month periods ended September 30, 2014 and September 30, 2013. Through September 30, 2014, no milestone obligations were incurred under the agreement.

Amended and Restated License Agreement

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

The Company is currently in compliance with these milestone requirements. During the term of the agreement, the Company is not obligated to make annual license or maintenance payments, but is obligated to pay to AmpliPhi all royalty and milestone payments that become due and payable by AmpliPhi to UPenn under AmpliPhi's agreement with UPenn as a result of the Company's exercise of the sublicense granted under the Company's agreement with AmpliPhi. This includes a low single-digit tiered percentage royalty on net sales of MYDICAR and any other product covered by the licensed patents sold by the Company, its affiliates or its sublicensees, and up to $850,000 in milestone payments upon the achievement of certain developmental and regulatory milestones related to MYDICAR and any other product covered by the licensed patents. Through September 30, 2014, $0.3 million of 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 is currently in compliance with these milestone requirements. The Company has the first right to prosecute and maintain the applicable patent family.

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

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

The Company recorded research and development expense related to license and annual maintenance fees under the agreement of $0.1 million in each of the nine month periods ended September 30, 2014 and September 30, 2013. Through September 30, 2014, no milestone obligations were incurred under the agreement.

Material Transfer and Exclusivity Agreement

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

The term of Servier's license to conduct the evaluation, or the evaluation period, will expire six months after Servier's initial receipt from the Company of the samples, provided that Servier may extend the evaluation period for up to an additional two months and may terminate the agreement at any time upon written notice to the Company.

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

License Agreement with Enterprise

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

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

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

Other License Agreements

The Company has entered into various license agreements pursuant to which the Company acquired certain intellectual property. Pursuant to each agreement the Company paid a license fee and reimbursed historical patent costs. Additionally, under each agreement, the Company may be required to pay annual maintenance fees, royalties, milestone payments and sublicensing fees. Each of the license agreements is generally cancelable by the Company, given appropriate prior written notice. Minimum annual payments to maintain these cancelable licenses total an aggregate of approximately $0.2 million and potential future milestone payments total an aggregate of approximately $3.3 million. The Company has recorded research and development expense related to license and annual maintenance fees under the agreements of $0.2 million and $0.2 million, respectively, for the nine months ended September 30, 2014 and September 30, 2013.

Through September 30, 2014, the Company has recorded research and development expense of $0.1 million related to milestone obligations incurred under the agreements.

Leases

The Company leases office space in San Diego, California under long-term operating leases that expire in October 2017 and September 2021. On July 1, 2014, the Company relocated its San Diego office to another location in San Diego and is subleasing the prior space. The rent expense for the three months and nine months ended September 30, 2014 was $94,000 and $136,000, respectively, and the rent expense for the three months and nine months ended September 30, 2013 was $21,000 and $63,000, respectively. Future minimum payments under the long-term operating leases net of contractual sublease payments total $2.9 million.

6. Stockholders' Equity (Deficit)

Common Stock and Common Stock Warrants

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

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

	Total	Nine months ended September 30, 2014	As of December 31, 2013
Gross proceeds (including over-allotment)	$50,600	$ 50,600	$ —
Underwriting discounts and commissions	(3,542)	(3,542)	—
Offering costs	(2,800)	(1,107)	(1,693)
Net proceeds	$44,258	$ 45,951	$ (1,693)

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

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

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

	Total	Subsequent to September 30, 2014	As of September 30, 2014
Gross proceeds (including option to purchase additional shares)	$43,700	$ —	$ 43,700
Underwriting discounts and commissions	(2,622)	—	(2,622)
Offering costs	(389)	(265)	(124)
Net proceeds	$40,689	$ (265)	$ 40,954

The following table summarizes the fully exercisable warrants outstanding for the purchase of common stock as of September 30, 2014 and December 31, 2013:

September 30, 2014	December 31, 2013	Exercise Price	Expiration Date
—	80	$224.82	January 2015
—	622	$12.49	October 2016
206,340	—	$5.61	October 2018
206,340	702		

Stock Options

Options granted under the Company's equity incentive plans generally expire no more than 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.

Prior Plans

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

2013 Equity Incentive Plan

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

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

	Options
Outstanding at December 31, 2013	1,543,667
Granted	1,181,973
Exercised	(163,087)
Cancelled	(35,486)
Outstanding at September 30, 2014	2,527,067

2013 Employee Stock Purchase Plan

In October 2013, the Company's stockholders approved the 2013 Equity Stock Purchase Plan (ESPP) which became effective in January 2014. Under the ESPP, an aggregate of 165,732 shares of common stock are initially authorized for issuance pursuant to purchase rights granted to the Company's employees or to employees of any of the Company's designated affiliates. The number of shares of common stock reserved for issuance will automatically increase on January 1 of each calendar year, from January 1, 2015 through January 1, 2023 by the least of (1) 1% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year, (2) 384,307 shares, or (3) a number determined by the Company's board of directors that is less than (1) and (2).

Stock-Based Compensation Expense

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Research and development	$391	$791	$1,175	$1,020
General and administrative	520	21	1,003	58
	$911	$812	$2,178	$1,078

As of September 30, 2014 the unrecognized compensation cost related to outstanding employee options was $9.3 million and is expected to be recognized as expense over approximately 3.2 years.

7. Term Loan and Security Agreement

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

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

The interest rate for each tranche will be calculated at a rate equal to the greater of either (i) 8.25% plus the prime rate as reported from time to time in The Wall Street Journal minus 5.25%, and (ii) 8.25%. Payments under the Loan Agreement are interest only until August 1, 2015 (which will be extended until February 1, 2016 if the Company achieves the Milestone on or before May 31, 2015) (the Amortization Date) followed by equal monthly payments of principal and interest through the scheduled maturity date on February 1, 2018 (the Loan Maturity Date). In addition, a final payment equal to $1,750,000 will be due at such time as the Loan is prepaid or becomes due and payable as specified in the Loan Agreement. The Company's obligations under the Loan Agreement are secured by a security interest in substantially all of its assets, excluding its intellectual property but including the proceeds from the sale, licensing or disposition of its intellectual property. The Company's intellectual property is also subject to customary negative covenants.

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

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

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

The following table summarizes the components of the term loan carrying value (in thousands):

	September 30, 2014
Obligations to lender: principal and final payment fee	$ 11,750
Debt discount	(1,839)
Carrying value	$ 9,911

8. Subsequent Event

Lonza Agreement

On October 31, 2014, the Company and Lonza Biologics, Inc. (Lonza) entered into a Facility Construction and Commercial Supply Agreement (the Lonza Agreement), pursuant to which the parties agreed to initiate detailed design planning (the Detailed Design) for the potential construction of a new commercial viral therapeutics facility in Portsmouth, New Hampshire for the manufacture of MYDICAR drug substance (AAV1/SERCA2a) (the Facility), and in exchange for an upfront $1,000,000 reservation fee payable by the Company to Lonza, Lonza agreed to reserve, for a period of time extendable on payment of specified reservation extension fees, the capital, property and labor resources necessary to enable the initiation of construction of the Facility within 75 days of receipt of notice of the Company's decision to exercise the construction trigger and commit to a long-term supply arrangement for MYDICAR (the Construction Trigger).

The Construction Trigger may not be exercised by the Company prior to completion of the Detailed Design for the Facility, which is currently expected to be completed by April 2015. If the Company exercises the Construction Trigger, Lonza would be obligated to purchase $10,000,000 worth of newly issued, unregistered shares of common stock of the Company and initiate construction of the Facility. In exchange, the Company would be obligated to (i) fund Lonza's construction of the Facility through time and event-triggered milestone payments secured by funds deposited by the Company into an escrow account upon exercise by the Company of the Construction Trigger, (ii) upon completion of the Facility, fund Lonza's costs for overhead, including personnel reserved for manufacture of MYDICAR at the Facility, and (iii) through such overhead funding arrangement order from Lonza a certain percentage of the Company's and its partners' annual global commercial supply of MYDICAR, subject to certain limits and adjustments.

The Lonza Agreement would continue in effect until the earlier of the sixth anniversary of the first approval of MYDICAR in the United States or European Union (First Approval) or expiration of the reservation period for construction of the Facility prior to the Company exercising the Construction Trigger, subject to earlier termination under specified circumstances set forth in the Lonza Agreement as described below. Additionally, if the Company exercises the Construction Trigger and is paying an agreed threshold for overhead for manufacture of MYDICAR at the Facility, the Company has the right to extend the term of the Lonza Agreement for an additional three years upon notice provided to Lonza between the third and fourth anniversary of the First Approval. The Company

has the right to terminate the Lonza Agreement (i) immediately upon notice to Lonza at any time prior to exercise of the Construction Trigger; (ii) upon 90 days' notice to Lonza if at any time the Company discontinues development and, if applicable, commercialization of MYDICAR as a result of regulatory, safety and/or efficacy concerns; or (iii) immediately upon notice to Lonza in the event of a certain specified material breaches of the Lonza Agreement by Lonza or Lonza's debarment. Additionally, each party may terminate the Lonza Agreement on uncured material breach of the Agreement by, or upon the insolvency or bankruptcy of, the other party, or in the event of a continuing force majeure preventing performance. Upon any termination following exercise of the Construction Trigger other than for material breach of the Lonza Agreement or Lonza's debarment, the Company is obligated to pay specified termination fees as set forth in the Lonza Agreement.

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, 2013, 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 success, cost and timing of our product development activities and clinical trials;
- our ability to obtain and maintain regulatory approval for MYDICAR, our companion diagnostic, and any of our future product candidates, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- our ability to obtain funding for our operations, including funding necessary to complete all clinical trials that may potentially be required to file a biologics license application, or BLA, and a Marketing Authorization Application, or MAA, for MYDICAR for the treatment of systolic heart failure;
- the commercialization of our product candidates and companion diagnostic, if approved;
- our plans to research, develop and commercialize our product candidates and companion diagnostic;
- our ability to attract collaborators with development, regulatory and commercialization expertise;
- our plans and expectations with respect to future commercial scale-up activities, including our expectation regarding the building of commercial manufacturing facilities for the production of MYDICAR;
- current and future agreements with Lonza Biologics, Inc. or its affiliates, or Lonza, and other third parties in connection with the commercialization of MYDICAR, our companion diagnostic and any other approved product;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- the rate and degree of market acceptance of our product candidates and companion diagnostic;
- regulatory developments in the United States and foreign countries;
- the performance of our third-party suppliers and manufacturers;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- our expectations regarding the period during which we qualify as an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or the JOBS Act; and
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates.

These forward-looking statements reflect our management's beliefs and views with respect to future events and are based on estimates and assumptions as of the filing date of this 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. We apply our leadership position in the field of gene therapy and calcium dysregulation to develop novel therapies for diseases with tremendous unmet medical needs and characterized by an underlying SERCA enzyme deficiency. MYDICAR, our most advanced product candidate, uses gene therapy to target SERCA2a, which is an enzyme that becomes deficient in patients with heart failure. MYDICAR utilizes a recombinant AAV1 serotype, which is a group of adeno-associated viruses, or AAVs, sharing specific antigens, to deliver the gene for the SERCA2a enzyme. We believe that our gene therapy approach to modulating SERCA2a overcomes the issues encountered by previous efforts and has the potential to provide transformative disease-modifying effects with long-term benefits in patients with advanced heart failure. In addition, we have recently 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 evaluating MYDICAR in a 250-patient randomized, double-blind, placebo-controlled international Phase 2b trial in patients with systolic heart failure, which we refer to as CUPID 2. We completed enrollment of CUPID 2 in February 2014 and expect to announce results in April 2015.

In April 2014, the FDA's Center for Biologics Evaluation and Research, or CBER, granted Breakthrough Therapy designation to MYDICAR for reducing hospitalizations for heart failure in patients who test negative for adeno-associated viral vector 1, or AAV1, neutralizing antibodies, are class III or IV heart failure patients under the New York Heart Association, or NYHA, classification system, and are not in immediate need of a left-ventricular assist device, or LVAD, or heart transplant. The Breakthrough Therapy program is intended to expedite drug development and review of innovative new drugs that are intended to treat serious or life-threatening diseases and for which preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on a clinically significant endpoint. MYDICAR was the third product candidate to receive this designation from CBER, and the designation indicates that the FDA has determined that the CUPID 1 trial provided preliminary clinical evidence that MYDICAR may demonstrate substantial improvement over available therapies in heart failure patients for which the designation was granted.

We have developed a companion diagnostic to identify patients who are AAV1 neutralizing antibody, or NAb, negative and therefore eligible for MYDICAR treatment. We believe approximately 40% of patients in the United States are AAV1 NAb negative. In an effort to expand the population of heart failure patients with systolic dysfunction that may be eligible for MYDICAR treatment, we are currently exploring whether plasma exchange can be used to remove AAV1 neutralizing antibodies from the circulation in advance of MYDICAR administration. We are currently in discussions with the FDA regarding a potential clinical trial using this procedure, and pending the outcome of these discussions we may initiate a clinical trial in 2015.

We are initially developing MYDICAR to treat patients with systolic heart failure. Heart failure caused by systolic dysfunction is characterized by a decreased contraction of the heart muscle. We are also developing MYDICAR for additional indications, including arteriovenous fistula, or AVF, maturation failure, and for the treatment of patients with advanced heart failure who are on a left-ventricular assist device, or LVAD. In addition, pending the outcome of our CUPID 2 trial, we expect to initiate a clinical trial in 2015 for the treatment of diastolic heart failure. This condition is also known as heart failure with preserved ejection fraction, which is caused by the inability of the heart to relax normally between contractions. MYDICAR has demonstrated activity in preclinical models of this condition.

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

In February 2014, we entered into a material transfer and exclusivity agreement with Les Laboratories Servier, or Servier, for the purpose of enabling Servier to conduct an evaluation of our small molecule compounds that modulate the SERCA2b enzyme. As part of this agreement, we granted Servier an option to enter into a license and research collaboration agreement for the joint collaboration, research and development of these compounds for the treatment of type 2 diabetes and other metabolic diseases, pursuant to which Servier may obtain an exclusive, royalty-bearing license to commercialize one or more of these compounds and any related products in the field of type 2 diabetes and other metabolic diseases outside of the United States.

In February 2014, we completed our initial public offering of 6,325,000 shares of common stock at an offering price of $8.00 per share, which included the exercise by the underwriters of their option to purchase 825,000 additional shares of common stock. We received gross proceeds of $50.6 million and incurred $6.3 million in issuance costs, resulting in net proceeds to us of $44.3 million.

In July 2014, we in-licensed world-wide rights to gene therapy applications for mSCF for treatment of cardiac ischemia from Enterprise Partners Management, LLC. Our initial focus with the program will be to generate clinically acceptable gene therapy vectors in support of potentially conducting a future clinical trial in patients who have suffered cardiac damage, as well as exploration of other potential applications.

In July 2014, we entered into a loan and security agreement with Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc. under which we may borrow up to $25.0 million in two tranches. We borrowed the first tranche of $10.0 million on August 1, 2014. We plan to use the proceeds of the agreement to support our product candidate pipeline and for working capital and general corporate purposes. The second tranche of up to $15.0 million can be drawn anytime through May 31, 2015, but only if data from our Phase 2b clinical trial for MYDICAR supports the continued development of MYDICAR for its Breakthrough Therapy designation to either a Phase 3 clinical trial or for registration for approval.

In August 2014, we completed an underwritten public offering of 4,600,000 shares of common stock at an offering price of $9.50 per share, which included the exercise by the underwriters of their option to purchase 600,000 additional shares of common stock. We received gross proceeds of $43.7 million and incurred $3.0 million in issuance costs, resulting in net proceeds to us of $40.7 million.

In October 2014, we entered into a facility construction and commercial supply agreement with Lonza pursuant to which we and Lonza agreed to initiate detailed design planning for the potential construction of a new commercial viral therapeutics facility in Portsmouth, New Hampshire for the manufacture of MYDICAR drug substance (AAV1/SERCA2a), and in exchange for an upfront $1,000,000 reservation fee payable by us to Lonza, Lonza agreed to reserve, for a period of time extendable on payment of specified reservation extension fees, the capital, property and labor resources necessary to enable the initiation of construction of the facility within 75 days of receipt of notice of our decision to exercise the construction trigger and commit to a long-term supply arrangement for MYDICAR, or the construction trigger. The construction trigger may not be exercised by us prior to completion of the detailed design for the facility, which is currently expected to be completed by April 2015. Our ability to exercise the construction trigger is subject to the terms and conditions of the agreement as well as other factors, and we may choose not to exercise the construction trigger even if we have the ability to do so as a result of a number of factors, certain of which are discussed under the heading “Risk Factors” in Part II, Item 1A of this Quarterly Report on From 10-Q.

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 September 30, 2014, we have funded our operations primarily through the sales of equity and debt securities totaling approximately $219.2 million, which includes the net proceeds from our 2014 public offerings.

We have incurred net losses in each year since our inception. As of September 30, 2014, we had an accumulated deficit of approximately $135.1 million. Substantially all 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 anticipate that our expenses will increase substantially if and as we:

- initiate, expand or accelerate preclinical and clinical development activities for our lead product candidate, MYDICAR, including with respect to clinical trials of MYDICAR for systolic heart failure (including our ongoing CUPID 2 trial), preclinical and potential clinical activities to evaluate MYDICAR for the treatment of AVF maturation failure, our preclinical activities and planned initial trial of MYDICAR in end-stage renal disease patients undergoing surgery for AVF creation, the LVAD trial, our planned AAV1 NAb positive trial and viral shedding trial, investigation of the feasibility of plasma exchange in removing AAV1 neutralizing antibodies in advanced heart failure patients just prior to treatment with MYDICAR and potential clinical trials of MYDICAR for the treatment of diastolic heart failure and other indications;
- further develop the manufacturing process for our product candidates, including commercial scale-up, and validation and automation of our companion diagnostic;
- change or add manufacturers or suppliers;

- 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 any other product candidate that successfully completes clinical trials;
- seek regulatory and marketing approvals for our companion diagnostic;
- 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;
- maintain, expand and protect our intellectual property portfolio;
- make milestone or other payments under any in-license or collaboration agreement;
- attract and retain skilled personnel;
- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with any of the above.

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

Financial 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 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 investigator grants, patient screening, laboratory work, clinical trial material management and statistical compilation and analysis;
- costs related to acquiring and manufacturing clinical trial materials and preparing for commercialization, including continued testing such as process validation and stability of drug substance and drug product;
- costs related to compliance with regulatory requirements; and
- payments related to licensed products and technologies.

From our inception through September 30, 2014, we have incurred approximately $107.6 million in research and development expenses, of which we estimate $101.9 million relates to our development of MYDICAR. We plan to increase our research and development expenses for the foreseeable future as we continue to develop MYDICAR for the treatment of systolic heart failure and additional indications and further advance the development of our companion diagnostic and other product candidates. 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, and costs related to acquiring and manufacturing clinical trial materials. We typically use our employee and infrastructure resources across multiple research and development programs.

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

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

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

MYDICAR

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

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

Small Molecule Program

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

Stem Cell Factor Program

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

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for employees in executive, 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, the cost of various consultants, occupancy costs and information systems costs.

Other Income (Expense)

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

Critical Accounting Policies and Estimates

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

While our significant accounting policies are 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 June 30, 2014, there had been no material adjustments to our prior period estimates of accrued expenses for clinical trials. However, due to the nature of estimates, we cannot assure you that we will not make changes to our estimates in the future as we become aware of additional information about the status or conduct of our clinical trials.

Stock-Based Compensation

Stock-based compensation expense represents the grant date fair value of employee stock option grants recognized over the requisite service period of the awards (usually the vesting period) on a straight-line basis, net of estimated forfeitures. For stock option grants with performance-based milestones, the expense is recorded over the remaining service period after the point when the achievement of the milestone is probable or the performance condition has been achieved.

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

We estimate the fair value of our stock 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 September 30, 2014 and 2013

The following table summarizes our results of operations for the three months ended September 30, 2014 and 2013 (in thousands):

	Three Months Ended September 30,		Increase / (Decrease)
	2014	2013	
Research and development	$ 5,316	$ 4,571	$ 745
General and administrative	2,815	952	1,863
Total other (expense) income	(227)	70	(297)

Research and Development Expenses. Research and development expenses were $5.3 million and $4.6 million for the three months ended September 30, 2014 and 2013, respectively. The increase of approximately $0.7 million was due primarily to an increase of $1.0 million in expenses incurred during the third quarter of 2014 associated with drug substance manufacturing scale-up, $0.5 million in compensation related to an increase in headcount and $0.2 million in license fees offset by a decrease of $0.6 million in clinical costs due to the completion of enrollment in our CUPID2 trial in the first quarter of 2014 and $0.4 million in stock-based compensation due to the termination of certain consulting contracts in 2013. We expect that our overall research and development expenses will continue to increase in 2014 as we initiate additional clinical trials and continue scale-up activities.

General and Administrative Expenses. General and administrative expenses were $2.8 million and $1.0 million for the three months ended September 30, 2014 and 2013, respectively. The increase of approximately $1.9 million was due primarily to an increase of $0.6 million in compensation related to an increase in headcount, $0.5 million in stock-based compensation and $0.3 million in business development and $0.5 million in patent and other costs. We expect that our general and administrative expenses will increase as we continue to operate as a public company. We believe that these increases will likely include increased costs for director and officer liability insurance, costs related to the hiring of additional personnel to support product commercialization efforts and increased fees for outside consultants, attorneys and accountants. We also expect to incur increased costs to comply with corporate governance, internal controls, investor relations and disclosures, and similar requirements applicable to public companies.

Other (Expense) Income. Other expense was $0.2 million and other income was $0.1 million for the three months ended September 30, 2014 and 2013, respectively. Other expense for the three months ended September 30, 2014 consisted primarily of $0.3 million of expense related to the accretion of debt discount and interest charges on our term loan offset by $44,000 in interest and other income. Other income for the three months ended September 30, 2013 consisted primarily of interest income and a foreign currency exchange gain.

Comparison of the Nine Months Ended September 30, 2014 and 2013

The following table summarizes our results of operations for the nine months ended September 30, 2014 and 2013 (in thousands):

	Nine Months Ended September 30,		Increase / (Decrease)
	2014	2013	
Research and development	$15,515	$11,707	$ 3,808
General and administrative	6,545	2,280	4,265
Total other (expense) income	(452)	75	(527)

Research and Development Expenses. Research and development expenses were $15.5 million and $11.7 million for the nine months ended September 30, 2014 and 2013, respectively. The increase of approximately $3.8 million was due primarily to an increase of $2.9 million in expenses incurred during the first nine months of 2014 associated with manufacturing scale-up and $0.9 million in compensation related to an increase in headcount. A decrease of $0.8 million decrease in clinical costs due to the completion of enrollment in our CUPID2 trial in the first quarter of 2014 was offset by an increase in non-clinical expenses and stock-based compensation. We expect that our overall research and development expenses will continue to increase in 2014 as we initiate additional clinical trials and continue scale-up activities.

General and Administrative Expenses. General and administrative expenses were $6.5 million and $2.3 million for the nine months ended September 30, 2014 and 2013, respectively. The increase of approximately $4.3 million was due primarily to an increase of $1.4 million in compensation related to an increase in headcount, $0.9 million in fees associated with being a public company, $0.9 million in stock-based compensation, $0.4 million in business development expenses and $0.7 million in travel and other personnel related costs. We expect that our general and administrative expenses will increase as we continue to operate as a public company. We believe that these increases will likely include increased costs for director and officer liability insurance, costs related to the hiring of additional personnel to support product commercialization efforts and increased fees for outside consultants, attorneys and accountants. We also expect to incur increased costs to comply with corporate governance, internal controls, investor relations and disclosures, and similar requirements applicable to public companies.

Other (Expense) Income. Other expense was $0.5 million and other income was $0.1 million for the nine months ended September 30, 2014 and 2013, respectively. Other expense for the nine months ended September 30, 2014 consisted primarily of $0.3 million of expense related to the accretion of debt discount and interest charges on our term loan and $0.2 million related to an increase in the fair value of the warrant liability prior to conversion to equity upon the closing of our initial public offering. Other income for the nine months ended September 30, 2013 consisted primarily of interest income and a foreign currency exchange gain.

Liquidity and Capital Resources

We have incurred net losses each year since our inception and as of September 30, 2014, we had an accumulated deficit of approximately $135.1 million. We anticipate that we will continue to incur net losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may obtain through one or more public or private equity offerings, debt financings, government or other third-party funding, strategic alliances and licensing or collaboration arrangements.

Since our inception through September 30, 2014, we have funded our operations primarily through the sale of our equity and debt securities. As of September 30, 2014, we had cash, cash equivalents and investments of approximately $95.1 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Our initial public offering completed in February 2014 resulted in net proceeds to us of $44.3 million after deducting underwriting discounts and commission and offering expenses payable by us, including $1.7 million in offering costs paid by us prior to December 31, 2013. The outstanding principal and accrued interest thereon under our outstanding convertible promissory notes converted into shares of our common stock upon the closing of our initial public offering. On July 31, 2014, we entered into a Loan Agreement with a lender under which we can borrow up to $25.0 million in two tranches. On August 1, 2014, we borrowed the first tranche in the amount of $10.0 million and received $9.7 million, net of fees, prior September 30, 2014 and paid $0.1 million in fees after September 30, 2014. Our underwritten public offering completed in August 2014 resulted in net proceeds to us of $40.7 million after deducting underwriting discounts and commission and offering expenses payable by us, including $0.3 million in offering costs paid by us after September 30, 2014.

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

	Nine Months Ended September 30,	
	2014	2013
Net cash provided by (used in):		
Operating activities	$(19,374)	$(11,377)
Investing activities	(71,010)	8,168
Financing activities	96,848	(677)
Net increase (decrease) in cash and cash equivalents	$ 6,464	$ (3,886)

Operating activities. Net cash used in operating activities of $19.4 million during the nine months ended September 30, 2014, was primarily a result of our net loss of $22.5 million. The primary difference between our net loss and our cash used in operating activities was $2.2 million of stock-based compensation, $0.2 million related to the change in fair value of the warrant liability, $0.2 million of non-cash interest related to the accretion of debt discount on our current and prior debt arrangements, $0.2 million amortization of premiums paid on investment securities, $0.2 million of changes in our operating assets and liabilities and $0.1 million of depreciation expense. In connection with our initial public offering which was completed in February 2014, the warrant liability was reclassified to additional paid-in capital, the outstanding principal and accrued interest on our convertible debt were converted into shares of our common stock and the unamortized debt discount related to the convertible debt was charged to expense.

Net cash used in operating activities of $11.4 million during the nine months ended September 30, 2013 was primarily a result of our net loss of $13.9 million. The primary difference between our net loss and our cash used in operating activities was $1.1 million of changes in our operating assets and liabilities, $1.1 million in stock-based compensation and $0.3 million in interest expense related to the amortization of discounts and premiums paid on investment securities and other non-cash expenses.

Investing Activities. Net cash (used in) provided by investing activities of $(71.0) million and $8.2 million during the nine months ended September 30, 2014 and 2013, respectively, was primarily a result of the purchases and maturities of investments. In 2014, $0.5 million was also used to purchase property and equipment.

Financing Activities. Net cash provided by financing activities of $96.8 million during the nine months ended September 30, 2014 consisted primarily of $94.3 million in proceeds received and $7.4 million in costs paid in connection with our public offerings, $9.7 million in net borrowings under our term loan, $0.1 million in proceeds upon the exercise of warrants in exchange for common stock and $0.1 million in proceeds from the sale of shares under our employee stock purchase plan.

Future Funding Requirements

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

Based upon our current operating plan, we believe that our existing cash, cash equivalents and short-term investments, will enable us to fund our operations through 2015. We intend to use our existing cash, cash equivalents and short-term investments to fund development activities, including commercial manufacturing capabilities and clinical activities related to the completion of our CUPID 2 trial, MYDICAR for the treatment of systolic heart failure in patients with existing LVADs and AVF maturation failure, internal salaries and external costs related to completion of our CUPID 2 trial and the remainder to fund working capital, including general operating expenses. We have based our estimates on assumptions that may prove to be wrong, and we may use our available

capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the development of our product candidates.

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

- the progress, costs, results and timing of our CUPID 2 trial, and the clinical development of MYDICAR for other potential indications;
- the willingness of the FDA to accept CUPID 2, as well as our other completed and planned preclinical studies and clinical trials and other work, as the basis for review and regulatory approval of MYDICAR for the treatment of systolic heart failure and for other potential indications;
- the outcome, costs and timing of seeking and obtaining FDA and any other regulatory approvals;
- the costs associated with establishing and maintaining commercial manufacturing capabilities;
- the costs associated with, establishing and maintaining product commercialization capabilities;
- the number and characteristics of product candidates that we pursue, including our product candidates in feasibility and preclinical development;
- the ability of our product candidates to progress through clinical development successfully;
- our need to expand our research and development activities;
- the costs of acquiring, licensing or investing in businesses, products, product candidates and technologies;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- our need and ability to hire additional management and scientific, medical and sales personnel;
- the effect of competing technological and market developments; and
- our need to implement additional internal systems and infrastructure, including financial and reporting systems.

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

Contractual Obligations and Commitments

As of September 30, 2014, there were no material changes, outside of the ordinary course of business, in our outstanding contractual obligations from those disclosed within “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” as contained in our Annual Report, other than the addition of the following contractual obligations (in thousands):

	Payments due by period				
	Total	Less than 1 year	1 – 3 Years	3 – 5 Years	More than 5 years
Obligations under term loan (1)	$13,929	$ 836	$7,680	$5,413	$ —
Operating lease obligation relating to facility (2)	2,848	345	786	833	884
Total	$16,777	$1,181	$8,466	$6,246	$ 884

(1) Consists of 10.0 million term loan borrowed by us on August 1, 2014 under our loan and security agreement with Hercules

Technology III, L.P. and Hercules Technology Growth Capital, Inc. dated July 31, 2014. A final payment equal to $1.8 million will be due at such time as all amounts borrowed under the loan and security agreement are prepaid or become due and payable. The term loan has a scheduled maturity date of February 1, 2018.

(2) Consists of facility lease encompassing 10,908 square feet of office space that expires in September 2021.

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

Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term duration of our investment portfolio and the low-risk profile of our investments, we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a sudden change in market interest rates on our investment portfolio. A 10% change in interest rates on September 30, 2014 would not have had a material effect on the fair market value of our portfolio.

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.

Our balance sheet as of September 30, 2014 includes cash and cash equivalents of $0.1 million denominated in euros through our wholly-owned subsidiary, Celladon Europe B.V., or Celladon Europe. The majority of payments made by Celladon Europe are denominated in euros. Such payments have not been material in any period since our inception. 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 nine months ended September 30, 2014. A 10% change in the euro-to-dollar exchange rate on September 30, 2014 would not have had a material effect on our results of operations or financial condition.

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

Item 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 September 30, 2014, 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 September 30, 2014 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 (*) 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 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 $22.5 million for the nine months ended September 30, 2014. As of September 30, 2014, we had an accumulated deficit of approximately $135.1 million. Our prior losses, combined with expected future losses, have had and may continue to have an adverse effect on our stockholders' equity and working capital.

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

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

- initiate, expand or accelerate preclinical and clinical development activities for our lead product candidate, MYDICAR, including with respect to clinical trials of MYDICAR for systolic heart failure (including our ongoing CUPID 2 trial), preclinical and potential clinical activities to evaluate MYDICAR for the treatment of AVF maturation failure, including a planned initial trial of MYDICAR in end-stage renal disease patients undergoing surgery for AVF creation, the LVAD trial, our planned AAV1 NAb positive trial and viral shedding trial, investigation of the feasibility of plasma exchange in removing AAV1 neutralizing antibodies in advanced heart failure patients prior to treatment with MYDICAR and potential clinical trials of MYDICAR for the treatment of diastolic heart failure and other indications;
- further develop the manufacturing process for our product candidates, including commercial scale-up, and validation and automation of our companion diagnostic;
- 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 any other product candidate that successfully completes clinical trials;

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

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

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

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

- completing research and preclinical and clinical development of our product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical trials;
- developing a sustainable, scalable, reproducible, and transferable manufacturing process for our vectors and product candidates;
- automating, validating and seeking and obtaining regulatory approvals for our companion diagnostic on a timely basis;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and, if approved, the market demand for our product candidates;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either by establishing a sales force, marketing and distribution infrastructure, or by collaborating with a partner;
- obtaining market acceptance of any approved products and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- identifying and validating new product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

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

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

Under our loan and security agreement with Hercules Technology Growth Capital, Inc. and its affiliate Hercules Technology III, L.P., which we refer to collectively as Hercules or the Lenders, in August 2014 we borrowed $10.0 million from the Lenders and we have the option to borrow up to $15.0 million through May 31, 2015, subject to the satisfaction of certain funding conditions related to our clinical development of MYDICAR. The loan agreement requires us to comply with restrictive covenants, including restrictive covenants that limit our ability to incur additional indebtedness; encumber the collateral securing the loan agreement; acquire, own or make investments; repurchase or redeem stock or other equity securities; declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest; transfer a material portion of our assets; acquire other businesses; or merge or consolidate with or into any other business organization. If we are unable to satisfy the conditions to borrow additional amounts, we may not be able to draw-down additional funds from the loan agreement. Moreover, an uncured breach of any of the covenants or other event of default under the loan agreement could lead to an event of default under the loan agreement. If any event of default occurs, then outstanding amounts under the loan agreement may become due and payable immediately, but we may not have access to such amounts on reasonable terms or at all, which could harm our liquidity, business, financial condition, operating results and prospects.

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

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

We are currently advancing our lead product candidate, MYDICAR for the treatment of systolic heart failure, through clinical development and other product candidates through preclinical development. Developing products is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates in clinical trials.

Our operations have consumed substantial amounts of cash since inception. As of September 30, 2014, our cash, cash equivalents and investments were $95.1 million. Our research and development expenses were $15.5 million and $11.7 million for the nine months ended September 30, 2014 and 2013, respectively. We plan to develop MYDICAR for additional indications including treatment of AVF maturation failure and for the treatment of patients with advanced heart failure who are on an LVAD. In addition, we are investigating the feasibility of using plasma exchange in removing AAV1 Nabs in advanced heart failure patients prior to administration of MYDICAR. Also, we are currently planning to initiate a clinical trial in 2015 for the treatment of diastolic heart failure, a condition caused by the inability of the heart to relax normally between contractions, if our early development activities and CUPID 2 data warrant. We believe that our existing cash, cash equivalents and investments will enable us to fund our operations through 2015. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates and companion diagnostic, as well as to further develop MYDICAR for additional indications, such as the treatment of patients with end-stage renal disease on hemodialysis undergoing surgery for AVF creation, the treatment of patients with advanced heart failure who are on an LVAD, and to potentially develop MYDICAR for diastolic heart failure. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

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 companion diagnostic. 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 our companion diagnostic, or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

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

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

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

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

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

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

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

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

In addition, the clinical trial requirements of the FDA, the EMA and other foreign regulatory authorities, and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. For example, the FDA has required us to conduct a safety and efficacy trial of patients with pre-existing NAbs to the AAV-based vectors used by MYDICAR as well as a viral shedding trial to determine the dissemination of our MYDICAR vector particles into the environment. At the moment, no gene therapy product has been approved in the United States and only one gene therapy product,

uniQure's Glybera, which received marketing authorization from the EMA in 2012, has been approved in Europe but has not yet been launched for commercial sale, 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. Approvals by the EMA may not be indicative of what the FDA may require for approval.

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

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

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

A key element of our strategy is to screen out patients with certain amounts of pre-existing NAbs to the AAV1 viral vector used by MYDICAR. We have developed a companion diagnostic that will be used in combination with MYDICAR to help us better identify those patients that may benefit from treatment with MYDICAR. Although we are currently exploring the feasibility of using a plasma exchange procedure to remove AAV1 neutralizing antibodies from advanced heart failure patients to enable their treatment with MYDICAR, the FDA may not permit this procedure to be investigated in certain patient populations, and such a procedure may ultimately prove to be unsafe, ineffective or cost prohibitive, and we cannot predict with certainty when, if ever, such a procedure could successfully be implemented on a broad basis or whether such a procedure would be covered for reimbursement by third-party payors. Accordingly, we will be dependent on such companion diagnostic, both during our clinical trials and in connection with any future commercialization of MYDICAR for systolic heart failure or for other indications. We expect that we will enter into a strategic alliance with a third party for the automation and commercialization of our companion diagnostic. We and any of our future collaborators may encounter difficulties in developing the companion diagnostic for commercial application, including issues in relation to automation, selectivity/specificity, analytical validation, reproducibility, or clinical validation of such companion diagnostic. Companion diagnostics are subject to regulation by the FDA, the EMA and other foreign regulatory authorities as medical devices and require separate regulatory clearance or approval prior to 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 administration components used in the cardiac catheterization laboratory are regulated as combination products. These include products where two or more separate products are packaged together (e.g., drug and device products); or a product packaged separately but intended for use only with an approved, individually specified product where both are required to achieve the intended use of the proposed product. MYDICAR will include labelling that specifies certain administration products, and the labeling of some of the administration products may need to be changed, e.g., to reflect a change in intended use, which revisions could delay our ability to commercialize MYDICAR.

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

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit patients to participate in testing our product candidates as well as completion of required follow-up periods. If patients are unwilling to participate in our gene therapy trials because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting trials and obtaining regulatory approval of potential products may be delayed. If there are delays in accumulating the required number of clinical events in trials where clinical events are a primary endpoint, such as our CUPID 2 trial, 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:

- 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. Although we did not experience difficulties enrolling patients in our 250-patient CUPID 2 trial, we may experience difficulties enrolling the requisite number of patients for subsequent clinical trials, including additional trials that may be required by the FDA for the approval of MYDICAR. For example, one significant obstacle to the timely recruitment and enrollment of a sufficient number of eligible patients in a Phase 3 trial of MYDICAR, if required, is the high prevalence of certain pre-existing NAbs to the viral vector used by MYDICAR, with, we believe, approximately 60% of potential patients in the United States exhibiting these antibodies. In other countries, such as Poland, the prevalence of pre-existing AAV1 NAbs is significantly higher. These antibodies neutralize the effectiveness of AAV-based vectors, such as MYDICAR, and although we are able to prescreen for the presence of these antibodies, the high prevalence of these antibodies in humans reduces the pool of available trial participants.

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

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

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

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

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

- delays in raising, or inability to raise, sufficient capital to fund the planned clinical trials;
- delays in reaching a consensus with regulatory agencies on trial design;
- 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 approval or commercial scale-up, 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.

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

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

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

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

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

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

Trial designs and results from previous trials, including the results from our CUPID 1 and ongoing CUPID 2 trial, are not necessarily predictive of our future clinical trial designs or results. In addition, our CUPID 1 and CUPID 2 trials had a combined enrollment of 301 patients, which may be fewer than the number of patients we may need to enroll in a Phase 3 trial for MYDICAR, if such trial is required by the FDA. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical trials.

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

The results from our CUPID 2 trial may not be sufficiently robust to support the submission of marketing approval for MYDICAR for the treatment of systolic heart failure. Before we submit MYDICAR for marketing approval, the FDA and the EMA may require us to conduct additional clinical trials.

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

In addition, in November 2013, the EMA indicated that if MYDICAR demonstrates a substantial and highly significant treatment effect in the advanced heart failure population, and no untoward effects attributable to MYDICAR are observed, a safety database of approximately 205 to 230 MYDICAR-treated subjects may be sufficient to assess safety and allow acceptance of an MAA for MYDICAR for the treatment of systolic heart failure. We therefore believe that, if the above conditions are met, a Phase 3 trial will not be required for marketing approval in Europe. It is possible, however, that the FDA or the EMA may not consider the results of our CUPID 2 trial to be sufficient for approval of MYDICAR for the treatment of systolic heart failure. If the FDA or the EMA requires additional studies, including Phase 3 trials, we will incur additional costs and delays in the marketing approval process, which would require us to expend more resources than we have available. In addition, it is possible that the FDA and the EMA may have divergent opinions on the elements necessary for a successful BLA and MAA, respectively, which may cause us to alter our development, regulatory and/or commercialization strategies.

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

As with many pharmaceutical and biological products, treatment with MYDICAR may produce undesirable side effects or adverse reactions or events. Although extensive preclinical safety and biodistribution testing conducted on MYDICAR and other AAV vectors, including the CUPID 1 trial of MYDICAR for systolic heart failure 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 timepoints after administration. In previous clinical trials involving AAV viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of an immunologic T-cell response against the vector capsid proteins. 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, also known as cancer, which could potentially enhance the risk of malignant transformation. Potential procedure-related events are similar to those associated with standard coronary intervention procedures, and may include vascular injury (e.g., damage to the femoral, radial, or brachial arteries at the site of vascular access, or damage to the coronary arteries) or myocardial injury. If any such adverse events occur, our clinical trials could be suspended or terminated and the FDA, the EMA or other foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. The product-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

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

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

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

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

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

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

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or supplements to a BLA submitted by us;

- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

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

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

Gene therapy remains a novel technology, with no gene therapy product approved to date in the United States and only one gene therapy product approved to date in Europe. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or, with respect to MYDICAR, in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion could have an adverse effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, in conducting clinical trials of MYDICAR in Europe, we are subject to environmental assessment legislation applicable to genetically modified organisms, or GMOs, which classifies the administration of GMOs to humans as a “deliberate release” of the GMO into the environment, thereby necessitating prior review and clearance by the applicable environmental assessment governing body. The level of scrutiny varies by country and some localities have additional requirements. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

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

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

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

If we are successful in commercializing MYDICAR or any other products, FDA and foreign regulatory agency regulations require that we report certain information about adverse medical events if those products may have caused or contributed to those

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

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

In May 2012, we obtained an SPA from the FDA for a potential Phase 3 pivotal clinical trial of MYDICAR. The FDA's SPA process creates a written agreement between the sponsoring company and the FDA regarding clinical trial design and other clinical trial issues, such as the trial endpoints, that can be used to support approval of a product candidate. The SPA is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of a 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 any change in the protocol for a clinical trial can invalidate an SPA or require the sponsor to submit an amendment.

Notably, CUPID 2 is substantially similar in design to the SPA Phase 3 protocol and uses the same primary efficacy endpoint. However, while we believe that the FDA's agreement in the SPA regarding the trial endpoints will support approval if the CUPID 2 trial is deemed a pivotal trial for purposes of BLA submission, the SPA does not directly apply to the CUPID 2 trial. There can also be no assurance that the FDA will ultimately consider our SPA to be binding, particularly on the CUPID 2 trial that we are conducting. The FDA could assert that additional data, including data obtained through one or more additional clinical trials, may be required to support a regulatory submission. 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, 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.

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

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

We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates and our companion diagnostic for our clinical trials. There are a small number of suppliers for certain capital equipment and raw materials that we use to manufacture our product candidates and our companion diagnostic. Such suppliers may not sell these raw materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these raw materials. Although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a product candidate or the reagent for our companion diagnostic to complete the clinical trial, 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 our viral vectors and product candidates for clinical testing purposes, and intend to continue to utilize Lonza as our sole or primary supplier for clinical trials. We also expect to rely upon third parties to produce materials required for the commercial production of our product candidates and companion diagnostic if we succeed in obtaining the necessary regulatory approvals. Because certain of our license agreements place restrictions on our ability to transfer or sublicense our intellectual property rights obtained under such agreements in connection with manufacturing activities, if any supplier we use requires a sublicense of our intellectual property rights for commercial manufacture of our viral vectors, product candidates or companion diagnostic, we may be unable to transfer or sublicense the requisite intellectual property rights, which may negatively impact our supply of our viral vectors, product candidates or companion diagnostic.

All entities involved in the preparation of therapeutic product for clinical trials or commercial sale, including our existing contract manufacturers, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with GMP and equivalent foreign standards. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's good laboratory practices, or GLPs, and 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 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 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 CROs are required to comply with the FDA's 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 or our CROs fail to comply with applicable GCPs, the clinical data generated in our future 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 test subjects 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. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which could materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

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

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

We may form strategic alliances, create joint ventures or collaborations or enter into licensing arrangements with third parties for the development and commercialization of our product candidates and companion diagnostic. We face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Any delays in entering into new strategic

partnership agreements related to our product candidates or companion diagnostic could delay the development and commercialization of our product candidates and reduce their competitiveness even if they reach the market. Moreover, we may not be successful in our efforts to establish a strategic partnership or other collaborative arrangement for any future product candidates or companion diagnostic because the potential partner may consider that our research and development pipeline is insufficiently developed to justify a collaborative effort, or that our product candidates and programs do not have the requisite potential to demonstrate safety and efficacy in the target population. Even if we are successful in establishing such a strategic partnership or collaboration, we cannot be certain that, following such a strategic transaction or license, we will be able to progress the development and commercialization of the applicable product candidates as envisaged, or that we will achieve the revenues that would justify such transaction.

Risks Related to Commercialization of our Product Candidates and Companion Diagnostic

We intend to rely on third parties to produce our viral vectors, product candidates and other key materials and for our companion diagnostic testing, but these manufacturers do not have 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 are in the process of transferring to Lonza. In particular, we are in the process of conducting a first demonstration batch at commercial scale of production (2,000-liter production bioreactor scale), which we expect to complete by the end of this year. However, there is no guarantee that the scale-up process will be able to be completed without complications or delay. Although we have entered into an agreement for the manufacture of our MYDICAR vector 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 our MYDICAR vector 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. Moreover, Lonza has not yet manufactured our MYDICAR vector on a commercial scale. 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 may be required to establish large-scale commercial manufacturing capabilities, either independently or with 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 no experience manufacturing pharmaceutical or biological products on a commercial scale and some of our suppliers, including Lonza, would need to increase their scale of production to meet our projected needs for commercial manufacturing of our product candidates, the satisfaction of which may not be met on a timely basis.

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

We similarly intend to enter into an agreement with a third-party partner for the commercial scale-up, automation and administration of our companion diagnostic. 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 characterization and validation activities necessary for commercial and regulatory approvals. If Lonza or any of our other manufacturing partners does not obtain such regulatory approvals, our commercialization efforts will be harmed. In addition, any significant disruption in our supplier relationships could harm our business. We source key materials from third parties, either directly through agreements with suppliers or indirectly through our manufacturers who have agreements with suppliers. There are a small number of suppliers for certain key materials that are used to manufacture our product candidates and companion diagnostic. Such suppliers may not sell these key materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have any control over the process or timing of the acquisition of these key materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these key materials.

Our ability and decision as to whether to exercise the construction trigger under our facility construction and commercial supply agreement with Lonza will depend on a number of factors. If we are unable to or determine not to exercise our rights under the agreement, Lonza will not proceed with the construction of the proposed Portsmouth facility and we will need to identify, negotiate terms for and secure alternate sources of commercial supply of MYDICAR in order to commercialize MYDICAR.

In October 2014, we entered into a Facility Construction and Commercial Supply Agreement with Lonza Biologics, Inc., or Lonza, pursuant to which we and Lonza agreed to initiate detailed design planning for the potential construction of a new commercial viral therapeutics facility in Portsmouth, New Hampshire for the manufacture of MYDICAR drug substance (AAV1/SERCA2a), and in exchange for an upfront $1,000,000 reservation fee payable by us to Lonza, Lonza agreed to reserve, for a period of time extendable on payment of specified reservation extension fees, the capital, property and labor resources necessary to enable the initiation of construction of the facility within 75 days of receipt of notice of our decision to initiate construction of the facility and commit to a long-term supply arrangement for MYDICAR, or the construction trigger. The construction trigger may not be exercised by us prior to completion of an agreed upon detailed design for the facility, which we currently expect may be completed by April 2015, but there can be no assurance that it will be completed on this timeframe, or at all. Our decision as to whether to exercise the construction trigger may be impacted by a number of factors, including, among others, the outcome of our CUPID 2 trial of MYDICAR, our expectations regarding clinical trial requirements and development timelines, and our perception of the prospects for commercialization of MYDICAR. Even if we view the results of our CUPID 2 trial as sufficiently favorable to support a decision to exercise the construction trigger, our ability to do so may be limited by a number of additional factors, including our ability to obtain financing on terms sufficient to fund our financial obligations under the agreement following exercise of the construction trigger. In addition, under our loan and security agreement with Hercules, we will be required to either obtain the consent of Hercules prior to exercising the construction trigger or pay off our outstanding loan under the agreement, and there is no guarantee that we will be able to do either within the timeframe available to us to exercise the construction trigger.

Even if we exercise the construction trigger, we may not realize the anticipated benefits under the agreement and may incur considerable losses and commercialization delays associated with the construction or planned construction of the facility. There can be no assurance that the construction of the facility will be completed within the budget we anticipate or in a timely manner, or in compliance with applicable laws and regulations. In connection with our exercise of the construction trigger, we will be required to commit to a long-term supply arrangement with Lonza that will involve minimum purchase obligations, which could inhibit our ability to secure an alternative commercial supply of MYDICAR on a cost-efficient basis upon entering into such arrangement. Additionally, establishing an alternative commercial manufacturing facility capable of meeting our anticipated commercial supply needs of MYDICAR drug substance would involve considerable additional time and expense that could materially harm our ability to successfully commercialize MYDICAR in a timely manner, or at all, as well as our financial position and results of operations.

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

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

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

Building an internal sales force involves many challenges, including:

- recruiting and retaining talented people;
- training employees that we recruit;

- setting the appropriate system of incentives;
- managing additional headcount; and
- integrating a new business unit into an existing corporate architecture.

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

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

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

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

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

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

We face competition both in the United States and internationally, including from major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Some of the pharmaceutical and biotechnology companies we know are developing gene therapies for heart failure that could potentially be competitive with or hinder the uptake of MYDICAR and change the standard of care for heart failure patients include Renova Therapeutics, NanoCor Therapeutics, Juventas Therapeutics, VentriNova, uniQure N.V. and Beat BioTherapeutics. In addition, many universities and private and public research institutes are active in our target disease areas.

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

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

Even if we are successful in achieving regulatory approval to commercialize a product candidate faster than our competitors, we may face competition from "biosimilars" due to the changing regulatory environment. In the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biological products that are demonstrated to be "highly similar," or "biosimilar," to or "interchangeable" with an FDA-approved biological product. This new pathway could allow competitors to reference data from biological products already approved after 12 years from the time of approval. In 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 others 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 others 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;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- acceptance by physicians and patients of the product as a safe and effective treatment;
- the potential and perceived advantages of such product candidate over alternative treatments, especially with respect to patient subsets that we are targeting with such product candidate;
- the existence of other gene therapy products utilizing an AAV vector, which potential patients may elect to take for other indications, thereby causing them to develop NAbs and making them ineligible to take MYDICAR;
- the safety of such product candidate seen in a broader patient group, including its use outside the approved indications;
- the cost of treatment in relation to alternative treatments;
- relative convenience and ease of administration;

- the strength of marketing and distribution support and timing of market introduction of competitive products;
- the effectiveness of our sales and marketing efforts;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage or reimbursement.

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

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

Successful sales of any approved product candidates depend on the availability of adequate coverage and reimbursement from third-party payors. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Sales of our product candidates will therefore depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, such as Medicare and Medicaid, private health coverage insurers and other third-party payors. 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.

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

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

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

More recently, on April 1, 2014, the Protecting Access to Medicare Act of 2014, or PAMA, was signed to law, which, among other things, significantly alters the current payment methodology under the CLFS. Under the new law, starting January 1, 2016 and every three years thereafter (or annually in the case of advanced diagnostic lab tests), applicable clinical laboratories must report laboratory test payment data for each Medicare-covered clinical diagnostic lab test that it furnishes during a time period to be defined by future regulations. The reported data must include the payment rate (reflecting all discounts, rebates, coupons and other price concessions) and the volume of each test that was paid by each private payor (including health insurance issuers, group health plans, Medicare Advantage plans and Medicaid managed care organizations). Beginning in 2017, the Medicare payment rate for each clinical diagnostic lab test will be equal to the weighted median amount for the test from the most recent data collection period. The payment rate will apply to clinical laboratory diagnostic tests furnished by a hospital laboratory if the test is separately paid under the hospital outpatient prospective payment system. Additionally, PAMA overrules reforms included in the 2014 final rule for the

Medicare Physician Fee Schedule that called for a process to permit CMS to adjust payments under the CLFS to account for technological changes in tools, machines, supplies, labor, instruments, skills, techniques and devices by which laboratory tests are produced and used beginning in 2015. Levels of reimbursement may be impacted by these initiatives and other current and future legislation, regulation or reimbursement policies of third-party payors in a manner that may harm the demand and reimbursement available for our products, including our companion diagnostic, which in turn, could harm our product pricing and sales.

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

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

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

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

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

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

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

- different regulatory requirements for approval of drugs and biological products in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- regulations under the U.S. Foreign Corrupt Practices Act and similar foreign anti-corruption laws;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;

- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

Risks Related to our Business Operations

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

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

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

****We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.***

As of September 30, 2014, we had 26 full-time employees. As we mature and expand our research and development and other pre-commercialization activities, we expect to expand our full-time employee base and to hire more consultants and contractors. In addition, we currently plan to commercialize MYDICAR, if approved, using an internal sales force to target selected cardiologists, heart failure specialists and third-party payors in the United States. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

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

We are exposed to the risk of fraudulent conduct or other illegal activity by our employees, independent contractors, principal investigators, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, promotion, sales, marketing and certain business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of

information obtained in the course of patient recruitment or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of business conduct and ethics applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

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

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws, including, without limitation, the federal Health Care Program Anti-Kickback Statute and the federal civil and criminal False Claims Acts. These laws may impact, among other things, our proposed promotional, sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include, but are not limited to:

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

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

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

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

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

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

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

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

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we

believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions. We do not currently carry biological or hazardous waste insurance coverage.

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

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

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

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

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

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, as well as rules subsequently implemented by the SEC and the NASDAQ Global Market have imposed various requirements on public companies. In July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Recent legislation permits smaller "emerging growth companies" to implement many of these requirements over a longer period and up to five years from the pricing of their initial public offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact (in ways we cannot currently anticipate) the manner in which we operate our business. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such coverage.

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 and our non-binding letter of intent 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. 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 Biosciences Corporation (including the patent rights sublicensed to us from the University of Pennsylvania, or 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.

Prior to our recently completed initial public offering, there was no public market for our common stock. We cannot assure you that an active, liquid trading market for our shares will develop or persist. You may not be able to sell your shares quickly or at a recently reported market price if trading in our common stock is not active.

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

- adverse results or delays in preclinical studies or clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- unanticipated serious safety concerns related to the use of any of our product candidates;
- reports of adverse events in other gene therapy products or clinical trials of such products;
- inability to obtain additional funding;
- any delay in filing an IND or BLA for any of our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND or BLA;
- failure to obtain regulatory and marketing approvals;
- sales or potential sales of our common stock by us or our stockholders in the future;

  failure to successfully develop 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 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 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 majority 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.

Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including exemption from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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

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

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

- variations in the level of expenses related to our product candidates, companion diagnostic or future development programs;
- if any of our product candidates receives regulatory approval, the level of underlying demand for these product candidates and wholesalers' buying patterns;
- addition or termination of clinical trials or funding support;
- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements.
- any intellectual property infringement lawsuit in which we may become involved; and
- regulatory developments affecting our product candidates or companion diagnostic or those of our competitors.

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

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

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

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

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

Certain holders of our securities are entitled to rights with respect to the registration of their shares under the Securities Act of 1933, as amended, or the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming

freely tradable without restriction under the Securities Act, except for shares held by our affiliates as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

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

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

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

We are at risk of securities class action litigation.

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

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

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

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

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

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

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

- authorizing the issuance of "blank check" preferred stock, the terms of which may be established and shares of which may be issued without stockholder approval;
- limiting the removal of directors by the stockholders;
- creating a staggered board of directors;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- eliminating the ability of stockholders to call a special meeting of stockholders; and
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings.

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

Our employment agreements with our executive officers 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 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

None.

Use of Proceeds

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

The net proceeds from our initial public offering have been invested in highly-liquid money market funds and investment grade corporate debt securities, pending their use. Our cash requirements for the first nine months of 2014 were primarily met with sources

of liquidity available to us at December 31, 2013, prior to our initial public offering. As of September 30, 2014, we have used approximately $1.6 million of the net proceeds from our initial public offering. There has been no material change in the expected use of the net proceeds from our initial public offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b), except we intend to dedicate all or a portion of the net proceeds received by us from the full exercise of the underwriters' option to purchase additional shares to a trial of MYDICAR for the treatment of AVF maturation failure.

Item 3. Defaults Upon Senior Securities

Not applicable.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

Not applicable.

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: November 12, 2014

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

Dated: November 12, 2014

/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	Assignment and License Agreement by and between the Registrant and Enterprise Management Partners, LLC dated July 18, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on July 21, 2014).
10.2	Loan and Security Agreement by and between the Registrant and Hercules Technology III, L.P. and Hercules Technology Growth Capital, Inc., dated as of July 31, 2014 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K filed with the SEC on August 5, 2014).
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

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)) 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. 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

c. 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: November 12, 2014

/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)) 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. 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

c. 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: November 12, 2014

/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: November 12, 2014

/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: November 12, 2014

/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.