TARGETED GENETICS CORP /WA/
Form 10-Q
May 07, 2009
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

x **QUARTERLY REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2009

¨ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
FOR THE TRANSITION PERIOD FROM _____ TO _____

COMMISSION FILE NUMBER: 0-23930

TARGETED GENETICS CORPORATION

(EXACT NAME OF REGISTRANT AS SPECIFIED IN ITS CHARTER)

Washington	**91-1549568**
(State of Incorporation)	**(I.R.S. Employer Identification No.)**

1100 Olive Way, Suite 100 Seattle, WA 98101

(Address of principal executive offices)(Zip Code)

(206) 623-7612

(Registrant s telephone number, including area code)

(Former name, former address and former fiscal year, if changed since last report)

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 the filing requirements for at least the past 90 days. Yes x 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 (§ 229.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ¨ No ¨

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, or a non-accelerated filer. See definition of accelerated filer and large accelerated filer in Rule 12b-2 of the Exchange Act. (check one):

Large accelerated filer ¨ Accelerated filer ¨ Non-accelerated filer ¨ Smaller reporting company x

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

Shares of Common Stock, par value $0.01 per share, outstanding as of May 1, 2009: 20,447,865

Table of Contents

TARGETED GENETICS CORPORATION

Quarterly Report on Form 10-Q

For the quarter ended March 31, 2009

TABLE OF CONTENTS

Table of Contents

PART I FINANCIAL INFORMATION

Item 1. *Unaudited Financial Statements*

TARGETED GENETICS CORPORATION

CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited)

	March 31, 2009	December 31, 2008
ASSETS		
Current assets:		
Cash and cash equivalents	$ 3,916,000	$ 5,216,000
Accounts receivable	257,000	317,000
Prepaid expenses and other	121,000	132,000
Total current assets	4,294,000	5,665,000
Property and equipment, net	1,177,000	1,285,000
Other assets		200,000
Total assets	$ 5,471,000	$ 7,150,000
LIABILITIES AND SHAREHOLDERS DEFICIT		
Current liabilities:		
Accounts payable and accrued expenses	$ 1,200,000	$ 1,735,000
Accrued employee expenses	321,000	368,000
Current portion of accrued restructure charges	812,000	656,000
Deferred revenue	1,776,000	1,227,000
Total current liabilities	4,109,000	3,986,000
Accrued restructure charges	6,766,000	6,934,000
Deferred rent		2,000
Commitments and contingencies		
Shareholders equity:		
Preferred stock, $0.01 par value, 10,000,000 shares authorized:		
Series A preferred stock, 180,000 shares designated, none issued and outstanding		
Common stock, $0.01 par value, 45,000,000 shares authorized, 20,447,198 shares issued and outstanding at March 31, 2009 and 20,238,865 shares issued and outstanding at December 31, 2008	204,000	202,000
Additional paid-in capital	317,050,000	316,900,000
Accumulated deficit	(322,658,000)	(320,874,000)
Total shareholders deficit	(5,404,000)	(3,772,000)
Total liabilities and shareholders deficit	$ 5,471,000	$ 7,150,000

See accompanying notes to condensed consolidated financial statements

1

Table of Contents

TARGETED GENETICS CORPORATION

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Unaudited)

	Three months ended March 31,	
	2009	**2008**
Revenue under collaborative agreements	$ 2,021,000	$ 2,499,000
Operating expenses:		
Research and development	2,120,000	3,946,000
General and administrative	1,361,000	1,889,000
Restructure charges	334,000	202,000
Total operating expenses	3,815,000	6,037,000
Loss from operations	(1,794,000)	(3,538,000)
Investment income	10,000	125,000
Net loss	$ (1,784,000)	$ (3,413,000)
Net loss per common share (basic and diluted)	$ (0.09)	$ (0.17)
Shares used in computation of basic and diluted net loss per common share	20,403,217	19,814,161

See accompanying notes to condensed consolidated financial statements

Table of Contents

TARGETED GENETICS CORPORATION

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited)

	Three months ended March 31,	
	2009	**2008**
Operating activities:		
Net loss	$ (1,784,000)	$ (3,413,000)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	114,000	157,000
Stock-based compensation	152,000	194,000
Changes in assets and liabilities:		
Accounts receivable	60,000	551,000
Prepaid expenses and other	11,000	(28,000)
Current liabilities	(582,000)	(872,000)
Deferred revenue	549,000	5,000
Accrued restructure expenses and deferred rent	(14,000)	(139,000)
Other non-current assets	200,000	
Net cash used in operating activities	(1,294,000)	(3,545,000)
Investing activities:		
Purchases of property and equipment	(6,000)	(34,000)
Proceeds from sale of investments		
Net cash used in investing activities	(6,000)	(34,000)
Financing activities:		
Payments under leasehold improvements and equipment financing arrangements		(1,000)
Net cash used in financing activities		(1,000)
Net decrease in cash and cash equivalents	(1,300,000)	(3,580,000)
Cash and cash equivalents, beginning of period	5,216,000	16,442,000
Cash and cash equivalents, end of period	$ 3,916,000	$ 12,862,000

See accompanying notes to condensed consolidated financial statements

Table of Contents

TARGETED GENETICS CORPORATION

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. Summary of Significant Accounting Policies

Basis of Presentation

The condensed consolidated financial statements included in this quarterly report have been prepared by Targeted Genetics Corporation, or Targeted Genetics, according to the rules and regulations of the Securities and Exchange Commission, or SEC, and according to accounting principles generally accepted in the United States of America, or GAAP, for interim financial statements. The accompanying balance sheet information as of December 31, 2008 is derived from our audited consolidated financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been omitted in accordance with the SEC s rules and regulations. Our condensed consolidated financial statements include the accounts of Targeted Genetics and our inactive, wholly owned subsidiaries, Genovo, Inc. and TGCF Manufacturing Corporation. There were no intercompany transactions for any of the periods included in this report. The condensed consolidated financial statements reflect, in the opinion of management, all adjustments (which consist solely of normal recurring adjustments) necessary to present fairly our financial position and results of operations as of and for the periods indicated.

We do not believe that our results of operations for the three months ended March 31, 2009 are necessarily indicative of the results to be expected for the full year or any other period.

The condensed consolidated financial statements included in this quarterly report should be read in conjunction with our audited consolidated financial statements and related footnotes included in our annual report on Form 10-K for the year ended December 31, 2008.

Our combined cash and cash equivalents totaled $3.9 million at March 31, 2009. We believe that our current financial resources and the cash we expect to receive from our collaborative partners and grants will only be sufficient to fund our operations until the end of the second quarter of 2009. This estimate is based on our ability to successfully perform planned activities and the receipt of expected funding under our collaborations and grants, and actual results could differ from our estimates. Unless we raise additional capital in the second quarter of 2009, we expect to begin the process of ceasing operations, seeking bankruptcy protection or otherwise winding up our business.

Fair Value

Our cash equivalents are recorded at cost, which approximates fair market value, and consist primarily of money market investments. Our money market investments are classified as Level 1 on the fair value hierarchy.

Recently Issued Accounting Standards

In December 2007, FASB s Emerging Issues Task Force, or EITF, reached a consensus on Issue No. 07-1, *Accounting for Collaborative Arrangements,* or EITF 07-1. EITF 07-1 defines collaborative arrangements and establishes reporting requirements for transactions between participants in a collaborative arrangement and between participants in the arrangement and third parties. It also establishes the appropriate income statement presentation and classification for joint operating activities and payments between participants, as well as the sufficiency of the disclosures related to these arrangements. EITF 07-1 is effective for fiscal years beginning after December 15, 2008. EITF 07-1 is effective for all of our existing collaborations in place after January 1, 2009. The adoption of EITF 07-1 did not have an effect on our financial position or results of operations for the first quarter of 2009. See Footnote 4 for further information.

Table of Contents

2. Accrued Restructure Charges

Restructure charges primarily include contract termination costs related to building lease activity and employee termination costs. We apply the provisions of Statement of Financial Accounting Standards, or SFAS, No. 146, *Accounting for Costs Associated with Exit or Disposal Activities,* or SFAS No. 146, as it relates to our facility in Bothell, Washington and record restructure charges on the operating lease for the facility as a result of our 2003 decision to discontinue use of the facility. Accrued restructure charges represent our best estimate of the fair value of the liability remaining under the lease and are computed as the present value of the difference between the remaining lease payments due less the net of sublease income and expense. These assumptions are periodically reviewed and adjustments are made to the accrued restructure charge when necessary. We record accretion expense as the difference between estimated cost and the present value of these costs using an assumed discount rate of 10%. Accretion expense is recorded on an ongoing basis through the end of the lease term in September 2015 and is reflected as a restructure charge in the accompanying consolidated statements of operations.

We also record employee termination benefit costs associated with restructuring our business or reductions in force as restructure charges. Employee termination benefit costs include one-time termination benefits that are not a part of an existing benefit arrangement, including severance payments, stock-based compensation charges related to modified stock awards and payments for post-employment medical coverage.

The table below presents a reconciliation of our accrued restructure liability for the three-month period ended March 31, 2009:

	Restructure Costs
December 31, 2008 accrued liability	$ 7,590,000
Charges related to employee termination benefits	146,000
Accretion expense	188,000
Cash payments	(346,000)
March 31, 2009 accrued liability	$ 7,578,000

Adjustments to the accrued restructure liability for the three months ended March 31, 2009 includes $146,000 of employee termination benefits and $188,000 of accretion expense for the period. The total of these charges and adjustments to the liability are reflected as restructure charges in the accompanying condensed consolidated statement of operations.

On February 3, 2009, we surrendered the Bothell facility to the landlord and ceased making rent payments for the facility, actions that constituted a default under the lease. We are seeking to negotiate a settlement with the landlord of our remaining obligations under the lease. There can be no assurance that we will be successful in negotiating a settlement and the landlord may terminate the lease as a result of our default and, among other potential remedies, seek to accelerate our obligations due under the lease. In March 2009, we forfeited a $200,000 deposit for the Bothell facility lease to the owner. From the time of our 2003 decision to discontinue use of the facility through March 31, 2009, we have recorded contract termination costs totaling $13.1 million for the Bothell facility. Under the terms of the current Bothell lease, we would incur an additional $2.8 million in accretion expense and would pay $10.4 million in rent through the expiration of the lease in September 2015, subject to reduction by the amount of lease payments received by the landlord if the facility is re-leased to another tenant. Through March 31, 2009, we have recorded $312,000 in employee termination benefits related to our restructuring to reduce expenses and realign and narrow our product development priorities, which we announced in November 2008.

We periodically evaluate our restructure estimates and assumptions and record additional restructure charges as necessary. Because restructure charges are estimates based upon assumptions regarding the timing and amounts of future events, significant adjustments to the accrual may be necessary in the future based on the actual outcome of events and as we become aware of new facts and circumstances. If we were to successfully negotiate a settlement with the facility landlord that reduces our remaining obligations under our Bothell lease or if we were to decide to resume use of the Bothell facility, a portion or all of any remaining accrued restructure charges related to the facility would be reversed. Such a reversal would be reflected as a reduction of restructure expenses and reflected in the period in which the contract is renegotiated or use is resumed. As of March 31, 2009, we are unable to determine the likelihood of any future adjustments to our accrued restructure charges.

Table of Contents

3. Equity

Stock Compensation

In May 2007, our shareholders approved our proposal to amend, restate and rename the Targeted Genetics Corporation 1999 Stock Option Plan into the Targeted Genetics Corporation Stock Incentive Plan, or the Stock Incentive Plan. The Stock Incentive Plan provides for the issuance of long-term incentive awards, or Awards, in the form of nonqualified and incentive stock options, or Options, stock appreciation rights, stock grants and restricted stock units. Effective January 2006, we adopted SFAS No. 123R, *Share-Based Payment,* which requires us to expense the fair value of share-based payments granted over the vesting period. This compensation expense includes: (a) compensation cost for all share-based stock options granted prior to, but not yet vested as of January 1, 2006, based on the grant-date fair value used for prior pro forma disclosures adjusted for forfeitures, and (b) compensation cost for all share-based payments granted after January 1, 2006, based on the grant-date fair value estimate in accordance with the provisions of SFAS No. 123R.

In the first quarter of 2009 we modified some outstanding restricted stock units. Under the revised restricted stock unit agreements the outstanding awards were not canceled upon termination of service and were immediately vested in full. Under SFAS No. 123R, these modified awards were revalued on the effective date of the modification and the entire stock-based compensation charge was recognized in full during the first quarter of 2009 as there is no longer a service requirement. We recorded expense of $58,000 relating to these awards for the three months ended March 31, 2009. This expense is reflected as restructure charges in the accompanying consolidated statement of operations.

The following table summarizes stock-based compensation expense related to employee stock options and restricted stock units under SFAS No. 123(R) for the three months ended March 31, 2009 and 2008:

	March 31,	
	2009	**2008**
Stock options:		
Research and development expense	$ 5,000	$ 29,000
General and administrative expense		14,000
Restricted stock units:		
Research and development expense	55,000	63,000
General and administrative expense	34,000	88,000
Restructure expense	58,000	
Total stock-based compensation expense	$ 152,000	$ 194,000

We estimate the fair value of each restricted stock unit on the date of the grant using the closing market price of our traded securities. We estimate the fair value of each stock option award on the date of the grant using the Black-Scholes-Merton option pricing model. There were no stock options granted during the three months ended March 31, 2009 or 2008. We apply an estimated forfeiture rate that we derive from historical forfeited shares.

4. Collaborative Agreements

We have entered into various product development relationships and license arrangements with pharmaceutical and biotechnology companies and non-profit organizations. Under these partnerships, we typically are reimbursed for research and development and manufacturing activities we perform. As part of these agreements we have received milestone and upfront payments and may receive additional milestone payments. Additionally, we may receive payments upon the occurrence of certain transactions involving covered products as well as royalties from product sales after commercialization.

Effective this quarter, we implemented EITF No. 07-01, *Accounting for Collaborative Arrangements,* or EITF 07-01, which prescribes that certain transactions between collaborators be recorded in the income statement on either a gross or net basis, depending on the characteristics of the collaboration relationship, and provides for enhanced disclosure of collaborative relationships. In accordance with EITF 07-01, we evaluated our collaborative agreements for proper income statement classification based on the nature of the underlying activity. Amounts due from our collaborative partners related to development activities are generally reflected as collaborative revenue and the costs incurred are reflected as research and development expense. We currently do not have any collaborations with commercialized products. The adoption of EITF 07-01 did not affect our financial position or results of operations.

Table of Contents

Revenues earned for the three months ending March 31, 2009 and March 31, 2008 under our research and development collaborations and license agreements are as follows:

	2009	2008
Celladon	$ 1,548,000	$ 798,000
NIAID	330,000	1,701,000
Department of Defense	143,000	
Total Collaborative Revenue	$ 2,021,000	$ 2,499,000

Celladon

In 2004, we entered into a collaboration agreement and manufacturing agreement with Celladon focused on the development of AAV-based drugs for the treatment of heart failure. In connection with the formation of this collaboration, certain of Celladon s investors purchased 395,413 shares of our common stock at $15.20 per share, resulting in net proceeds to us of $6.0 million. We recorded the proceeds as equity at the fair value of the common stock, which approximated market value. Under the collaboration agreement, we agreed to contribute up to $2.0 million to support these development activities and then to be reimbursed for efforts over that amount. We met this $2.0 million threshold during 2005. We were also entitled to receive milestone payments during the development of product candidates under the collaboration as well as royalties and manufacturing profits from the commercialization of product candidates developed under the collaboration. In February 2009, we and Celladon agreed to replace the prior collaboration and manufacturing agreements with a license agreement and new manufacturing agreement. Under the terms of the modified agreements, we granted Celladon exclusive use of certain proprietary AAV vector technology in a specified field relating to heart failure, agreed to manufacture Celladon s MYDICAR® product candidate for phase III clinical studies, at Celladon s expense, and agreed to transfer technology to enable Celladon to manufacture MYDICAR® in the future through contract manufacturing organizations or a commercial partner. In addition, we and Celladon agreed to a new milestone payment and royalty structure covering development and commercialization of products in the permitted field, and Celladon agreed to make payments to us in the event of specified strategic transactions involving Celladon. Celladon separately manages and funds the clinical trial costs of the heart failure program. Our current work plan with Celladon extends through July 2009.

National Institute of Allergy and Infectious Diseases

In 2005, we extended the scope of our HIV/AIDS vaccine program to include the developed world via a contract awarded by the National Institute of Allergy and Infectious Disease, or NIAID, to Nationwide Children s Hospital (formerly known as Columbus Children s Research Institute), or NCH, in collaboration with Children s Hospital of Philadelphia, or CHOP, and us. Under the original award the NIAID established a $22.0 million budget for the overall collaboration, of which they identified a subcontract budget of up to $18.2 million of funding over five years for our efforts for the development, manufacture and preclinical testing of vaccine candidates. Since 2005 investigators at CHOP and NCH completed the design of the vaccine candidates and we have manufactured the vectors for the clinical trials that are planned to be conducted in the U.S. The direct costs of any clinical trials will be borne by the NIAID and are not part of the contract. This NIAID-funded vaccine program complements work we performed under our vaccine program with International AIDS Vaccine Initiative. The NIAID awards funding under this program in annual installments. Total cumulative funding awarded to us under our subcontract is $15.8 million for the performance period through August 30, 2009, and we have recognized cumulative revenues of $10.9 million through March 31, 2009. In 2009 we expect to receive less NIAID funding and recognize less HIV/AIDS vaccine program revenue, as compared to 2008, as we wind down our portion of the development efforts and terminate our involvement in the program.

U.S. Department of Defense

In 2008, we entered into an agreement to develop a small-molecule based product candidate to treat amyotrophic lateral sclerosis in collaboration with John Engelhardt, Ph.D., at the University of Iowa and funded by a grant from the U.S. Department of Defense, or DOD. Under the award, the DOD has approved grant funding of up to $2.4 million for the reimbursement of research and development costs we incur during 2009.

Table of Contents

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

Forward-Looking Statements

This quarterly report on Form 10-Q contains forward-looking statements that involve risks and uncertainties. Forward-looking statements include statements about our cash resources and future financial condition, our ability to continue as a going concern, our ability to obtain additional funding, our listing on the Nasdaq Capital Market, our product development and commercialization goals and expectations, potential market opportunities, our plans for and anticipated results of our clinical development activities and the potential advantage of our technology and product candidates and other statements that are not historical facts. Words such as may, can be, may depend, will, believes, estimates, expects, anticipates, plans, projects, intends, or statements concerning potential or opportunity and other words of similar meaning or t negative thereof, may identify forward-looking statements, but the absence of these words does not mean that a statement is not forward-looking. In making these statements, we rely on a number of assumptions and make predictions about the future. Our actual results could differ materially from those stated in or implied by forward-looking statements for a number of reasons, including the risks described in the section Risk Factors in Part II, Item 1A of this quarterly report.

You should not unduly rely on these forward-looking statements, which speak only as of the date of this quarterly report. We undertake no obligation to publicly revise any forward-looking statement after the date of this quarterly report to reflect circumstances or events occurring after the date of this quarterly report or to conform the statement to actual results or changes in our expectations. You should, however, review the factors, risks and other information we provide in the reports we file from time to time with the SEC.

BUSINESS OVERVIEW

We are a clinical-stage therapeutic biotechnology company. We are at the forefront of developing, with the goal of commercializing, a new class of therapeutic products called gene therapeutics. We believe that a wide range of diseases may potentially be treated or prevented with gene therapeutics. In addition to treating diseases for which there is no treatment, we believe that there is a significant opportunity to use gene therapeutics to more effectively treat diseases that are currently treated using other therapeutic classes of drugs such as protein-based drugs, monoclonal antibodies or small molecule drugs.

Gene therapeutics consist of a delivery vehicle, called a vector, and genetic material. The role of the vector is to carry the genetic material into a target cell. Once delivered into the cell, the gene can express or direct production of the specific proteins encoded by the gene. Gene therapeutics may be used to treat disease by facilitating the normal protein production or gene regulation capabilities of cells. Gene therapeutics may be used to treat a disease state by enabling cells to produce more of a certain protein or different proteins than they would normally produce. Vectors can also be used to deliver specific genetic sequences that, once delivered and expressed as an interfering RNA molecule, or RNAi, can shut down or interfere with the production of disease-specific genes by messenger RNA, or mRNA.

We are a leader in the preclinical and clinical development of gene therapeutics based on adeno-associated viral, or AAV, vectors, and in the manufacture of AAV vectors. We have treated over 400 subjects in clinical trials using AAV-based gene therapeutic product candidates and, through our research and development activities, we have acquired expertise and intellectual property related to AAV-based gene therapeutic technologies. In addition, based on research developed by one of our collaborators to improve the delivery of AAV vectors, a new product opportunity emerged for a small molecule therapy to potentially treat neurological diseases associated with oxidative stress. We have applied our development expertise to this early-stage small molecule and in 2008 we initiated a preclinical program around that opportunity. As a result of these AAV- and small molecule-related efforts, we believe we have generated potential value through our development and manufacturing expertise, through the potential of our accumulated intellectual property portfolio and through our application of our expertise and intellectual property to promising product candidates.

In November 2008, we reprioritized our product development efforts and focused our internally funded efforts on ocular and neurological product candidates, including our first product development effort to evaluate the use of AAV to deliver expressed RNAi. This realignment focused our resources on creating near-term value balanced with the capabilities and resources currently available to us and our collaborators. We implemented this realignment to scale our operations down to match our projected financial resources and to focus our expertise and intellectual property on the programs we believe offer the most promise. As a result, our development efforts are currently focused on:

- a clinical-stage AAV-based product candidate for the treatment of Leber s congenital amaurosis, or LCA, developed with Robin Ali, Ph.D., our collaborator at the University College London/Moorfields Eye Hospital, or UCL/M. LCA

Table of Contents

is an ocular disease, one cause of which is a mutation in the RPE65 gene that leads to blindness. This product candidate is currently enrolling subjects in a Phase I/II dose escalation clinical trial funded through a grant awarded to Dr. Ali;

a preclinical AAV-based Huntington s disease, or HD, product candidate under development with our collaborator, Beverly Davidson, Ph.D., at the University of Iowa, or UI. HD is an incurable neurodegenerative disorder that results from mutations to the gene that codes for the huntingtin protein. Preclinical studies focused on identifying a clinical candidate continue at UI. Based on an ongoing review of the program development timeline and funding requirements for future development, combined with an assessment of partnering interest from other parties to support future development efforts, we are in the process of evaluating our involvement with this program going forward; and

a preclinical small-molecule-based product candidate to treat amyotrophic lateral sclerosis, or ALS, under development with our collaborator, John Engelhardt, Ph.D., at UI. ALS is a progressive neurodegenerative disease affecting the brain and spinal cord. Our 2009 efforts are funded by a grant to us from the U.S. Department of Defense, or DOD. We have an option with UI related to this program that expires in June 2009. The UI option will expire in June 2009 unless we are able to secure adequate funding to support the program moving forward.

We are leaders in the development and application of processes to manufacture potential products at a scale amenable to late-stage clinical development and expandable to large-scale commercial production, and we have established broad capabilities in applying our AAV-based gene therapeutic technologies to multiple product candidates and therapeutic indications. In late 2008 and early 2009, we analyzed the financial impact of continuing to support our internal manufacturing infrastructure compared to purchasing manufacturing services from contract manufacturing organizations, or CMOs. We determined that, based on our progress in developing a robust set of manufacturing processes, we could feasibly outsource our manufacturing needs rather than maintain the infrastructure costs of supporting our in-house manufacturing capability. In connection with this decision, in February 2009 we and Celladon Corporation agreed to conduct our previously agreed manufacturing campaign for Celladon s MYDICAR® congestive heart failure product candidate at our company facilities and, in parallel, transfer the manufacturing know-how and processes required to replicate our manufacturing and testing of MYDICAR® to third party CMOs. We plan to continue to maintain an internal knowledge base within the company to facilitate high-quality training and oversight of manufacturing information transfer to CMOs and provide for a high-level capability for continued development of new or improved manufacturing processes. However, we believe that we have built a very capable manufacturing unit, both in terms of facility and competency, and we may pursue retaining this infrastructure if we successfully raise the necessary funding to support such capability by mid-2009 or if the capability could be maintained by an acquirer of our manufacturing asset.

In February 2009, we replaced our collaboration and manufacturing agreements with Celladon with a license agreement and a new manufacturing agreement. Additionally, in early 2009, in connection with our realignment efforts, we began to terminate a portion of our subcontract with Children s Hospital of Philadelphia, or CHOP, and Nationwide Children s Hospital, or NCH, for an HIV/AIDS vaccine project funded by the National Institute of Allergy and Infectious Diseases, or NIAID, as our work on the program is nearing completion and the program is entering into clinical trials. We also continued to realign our intellectual property portfolio to focus on our current priorities, which included returning rights under licenses and/or cessation of prosecution of patents that are not specific to our current development program efforts. Based on the additional revenue expected from the new Celladon agreements, in combination with reduced infrastructure, external program support and intellectual property costs and other spending reductions, we believe we have extended our cash horizon through the second quarter of 2009.

Most of our expenses are related to our research and development programs, the conduct of preclinical studies and clinical trials and general and administrative support for these activities. We have financed the company primarily through proceeds from public and private sales of our equity securities, through cash payments received from our collaborative partners for product development and manufacturing activities, and through proceeds from the issuance of debt and loan funding under equipment financing arrangements. These financing sources have historically allowed us to maintain adequate levels of cash and cash equivalents but, particularly in the current market environment, they may not continue to do so.

As of March 31, 2009, our accumulated deficit totaled $322.7 million and our cash balance was $3.9 million. We believe that our current financial resources, together with the cash we expect to receive from our collaborative partners and grants, will only be sufficient to fund our operations through the end of the second quarter of 2009. Unless we raise additional capital by then, we expect to begin the process of ceasing operations, declaring bankruptcy or otherwise winding up our business. Given the short amount of time and the fact that we have so far been unable to secure additional financial resources notwithstanding our considerable efforts to date, we believe it is increasingly unlikely we will be able to secure additional financial resources in time.

As a result of the goodwill impairment charge we recognized in the fourth quarter of 2008, combined with accrued restructure charges totaling $7.6 million at March 31, 2009 our net worth has fallen to a deficit of $5.4 million at March 31, 2009, which is below the

9

Table of Contents

$2.5 million in shareholders equity required for continued listing in the NASDAQ Capital Market under Marketplace Rule 4310(c)(3). We do not meet the alternative continued listing requirements of $35.0 million in market value of listed securities or $500,000 of net income from continuing operations. On April 8, 2009, we received a letter from NASDAQ informing us of our non-compliance with Marketplace Rule 4310(c)(3). The letter stated that we had until April 23, 2009 to submit a plan to regain compliance and that, if the plan were to be accepted, we could be provided with up to 105 calendar days from April 8, 2009 to demonstrate compliance. On April 23, 2009, we submitted our compliance plan to NASDAQ.

CRITICAL ACCOUNTING POLICIES, ESTIMATES AND ASSUMPTIONS

There have been no material changes from the critical accounting policies, estimates and assumptions disclosed the section entitled *Management s Discussion and Analysis of Financial Condition and Results of Operations* in Item 7 of our annual report on Form 10-K for the year ended December 31, 2008.

RESULTS OF OPERATIONS

Revenue

Revenue decreased to $2.0 million for the three months ended March 31, 2009 as compared to $2.5 million for the same period in 2008. The decrease in revenue reflects a decrease in research and development and manufacturing activities under the NIAID-funded HIV/AIDS vaccine project in collaboration with CHOP and NCH, and is partially offset by an increase in research and development activities under the Celladon program and our work on the ALS program. We expect that our revenue for the remainder of 2009 will consist primarily of research and development revenue earned from our manufacturing and technology transfer efforts in support of our agreement with Celladon and our ALS effort funded by the DOD, which was initiated in the fourth quarter of 2008 and is expected to be completed in 2009. Our HIV/AIDS vaccine development revenue has fluctuated year to year depending upon the scope of development efforts underway. For 2009, we expect modest revenue contributions from our HIV/AIDS efforts as our internal HIV/AIDS efforts will wind down as the NIAID begins clinical trials of the product candidate.

Operating Expenses

Research and Development Expenses. Research and development expenses decreased to $2.1 million for the three months ended March 31, 2009 from $3.9 million for the same period in 2008. The decrease reflects lower pass-through outside services for our NIAID-funded HIV/AIDS vaccine subcontract, and lower clinical trial costs for our inflammatory arthritis program, as we have completed our Phase I/II clinical trial. Research and development costs under the Celladon heart failure program increased in the first three months of 2009, as compared to the same period in 2008, due to our manufacturing efforts in 2009.

The following table sets forth the allocation of total research and development costs between our programs that are in clinical development and those that are in research or preclinical stages of development:

	Three months ended March 31,	
	2009	**2008**
Programs in clinical development:		
Heart failure	$ 835,000	$ 503,000
Inflammatory arthritis	47,000	507,000
Indirect costs and other	659,000	921,000
Total clinical development program expense	1,541,000	1,931,000
Research and preclinical development program expense	579,000	2,015,000
Total research and development expense	$ 2,120,000	$ 3,946,000

Research and development costs attributable to programs in clinical development include the costs of salaries and benefits, outside services, materials and supplies incurred to support the clinical programs. Indirect costs allocated to clinical programs include facility and occupancy

costs, research and development administrative costs, and license and royalty payments. These costs are further allocated between clinical and preclinical programs based on relative levels of program activity. Celladon separately manages and funds the clinical trial costs of the heart failure program and, as a result, we do not include those costs in our research and development expenses.

Costs attributed to research and preclinical programs represent our earlier-stage development activities and include costs incurred for development activities for the NIAID-funded HIV/AIDS vaccine program under a subcontract with CHOP and NCH, costs incurred for our ALS project funded by the DOD, and costs incurred for other programs prior to their transition into clinical trials. Research and preclinical program expense also includes costs that are not allocable to a clinical development program,

Table of Contents

such as unallocated manufacturing infrastructure costs. Because we conduct multiple research projects and utilize resources across several programs, our research and preclinical development costs are not directly assigned to individual programs.

For purposes of reimbursement from our collaboration partners, we capture the level of effort expended on a program through our project management system, which is based primarily on human resource time allocated to each program, supplemented by an allocation of indirect costs and other specifically identifiable costs, if any. As a result, the costs allocated to programs identified in the table above reflect the relative costs of the program.

General and Administrative Expenses. General and administrative expenses decreased to $1.4 million for the three months ended March 31, 2009 from $1.9 million for the same period in 2008. The decrease reflects lower intellectual property costs resulting from our return of licensed patent rights and cessation of prosecution of patents that are not specific to our current development program efforts, lower employee costs resulting from our reductions in force, lower stock-based compensation charges and lower shareholder annual meeting-related costs. We expect our general and administrative expenses for the remainder of 2009 to decrease as compared to 2008 as a result of our cost-cutting efforts.

Restructure Charges. Restructure charges increased to $334,000 for the three months ended March 31, 2009 from $202,000 for the same period in 2008. This increase is primarily due to employee termination benefit costs related to the reductions in force implemented as part of our efforts to realign and narrow our product development priorities.

Other Income and Expense

Investment Income. Investment income reflects interest income earned on our short-term investments. Investment income decreased to $10,000 for the three months ended March 31, 2009 from $125,000 for the same period in 2008, primarily due to lower average cash balances and lower interest rates compared to 2008.

Liquidity and Capital Resources

We had cash and cash equivalents of $3.9 million at March 31, 2009, compared to $5.2 million at December 31, 2008. The decrease primarily reflects cash used in operations of $1.3 million.

Our primary sources of capital are public and private sales of our equity securities and cash payments received from our collaborative partners and through proceeds from the issuance of debt. To a lesser degree, we have also financed our operations through interest earned on our cash and, in the last two years, through license revenue. These financing sources have historically allowed us to maintain adequate levels of cash and cash equivalents but, particularly in the current market environment and given our inability to secure additional financial resources notwithstanding our considerable efforts to date, we believe they are increasingly unlikely to continue to do so. Our primary expenses are currently related to conducting the Celladon MYDICAR® manufacturing campaign and technology transfer, the development of our research and development programs, prosecution of our intellectual property interests, and general and administrative support for these activities.

Most of our revenue has been derived under collaborative research and development agreements relating to the development of our potential product candidates. We do not expect the revenue generated from our current or future collaborative research and development and manufacturing arrangements to be sufficient to fully fund the development and commercialization of our product candidates. As a result, even if we are able to secure additional financial resources in time to continue our operations, which we believe is increasingly unlikely, we do not expect to generate ongoing positive cash flow from our operations for the foreseeable future and our ability to generate any sustained positive cash flow is dependent upon our success at developing and commercializing our product candidates.

We will require substantial additional funding to continue our operations and to fund development and commercialization of our three primary product development programs, which are LCA, HD and ALS. While the LCA program, currently in clinical trials sponsored and funded by our collaborative partner UCL/M, will not require substantial funding or staff support from us in 2009, our HD collaboration with UI requires us to make annual license payments, to fund intellectual property prosecution and to fund certain product development efforts. In addition, our option agreement with UI related to the ALS program requires us, by June 30, 2009, to meet certain financial diligence requirements, including the financial capability to fund ALS research at UI, in order to exercise the option and take a license to the intellectual property at UI related to the program. Based on financing progress and anticipated product development timelines, in light of our current financial resources, we are in the process of evaluating our continued involvement in both the HD and ALS programs going forward. We will continue later-stage development of our inflammatory arthritis product candidate only if we receive funding from a partner or acquirer of the program.

We require additional financial resources to continue our operations past the second quarter of 2009. In April 2009, we further reduced our staff as the result of our inability to obtain additional funding in the first quarter of 2009. Unless we raise

11

Table of Contents

additional capital by the end of the second quarter of 2009, we expect to begin the process of ceasing operations, seeking bankruptcy protection or otherwise winding up our business. Even if we are able to extend our cash horizon, if we do not receive sufficient additional funding before we reach the end of our financial resources, we will nevertheless be forced to cease operations, seek bankruptcy protection or otherwise wind up our business.

Our current operating strategy is to carefully steward our available funds to advance our three primary programs while leveraging our development and manufacturing capabilities and intellectual property assets into additional capital-raising opportunities. Key to this strategy is the completion of a manufacturing campaign of cGMP materials for a Phase III clinical trial of MYDICAR® under our agreement with Celladon, both for the cash that it provides to support 2009 operations and for the value generated for us through fulfilling this contractual commitment. Our current work plan with Celladon extends through July 2009.

We must secure additional financial resources before the end of the second quarter of 2009 in order to continue our operations. Our near-term financing strategy includes leveraging our development and manufacturing capabilities and intellectual property assets into additional capital raising opportunities, and seeking capital through a wide variety of sources, including accessing the public and private capital markets and pursuing potential strategic transactions. In the capital markets today there is extreme competition for capital to fund biotechnology businesses that do not have product sales and do not have later stage products showing high levels of efficacy in Phase II clinical trials. Moreover, in the biotechnology industry there is a low level of success in clinical trials and our ability to raise capital depends in part on clinical trial success.

We are currently evaluating additional sources of financing that could involve one or more of the following, although we have been unable to secure additional financing notwithstanding our considerable efforts to date:

- strategic transactions, including mergers and acquisitions;
- selling or licensing our technology, product candidates or other assets;
- entering into additional product development or manufacturing collaborations;
- extending or expanding our current collaborations; and/or
- issuing equity or debt in the public or private markets.

Additional funding may not be available to us on reasonable terms, if at all. The capital markets have been experiencing extreme volatility and disruption for over a year, and the volatility and disruption have reached unprecedented levels in recent months. The scope and extent of this disruption in the capital markets could make it difficult or impossible to raise additional capital in public or private capital markets until conditions stabilize and conditions may not stabilize in the very short amount of time we have left before we reach the end of our financial resources and are forced to go out of business.

Item 4T. ***Controls and Procedures***

Evaluation of disclosure controls and procedures. Based on our management s evaluation, with the participation of our Chief Executive Officer and Chief Financial Officer, of the effectiveness of our disclosure controls and procedures as of the end of the period covered by this quarterly report, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures are effective in ensuring that information we are required to disclose in reports that we file or submit under the Securities Exchange Act of 1934, as amended, is recorded, processed, summarized and reported within the time periods specified in SEC rules and forms.

Changes in internal control over financial reporting. There was no change in our internal control over financial reporting that occurred during the period covered by this quarterly report that materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

12

Table of Contents

PART II OTHER INFORMATION

Item 1. Legal Proceedings

In July 2007, we were notified that a patient experienced a serious adverse event, or SAE, while enrolled in the clinical trial of tgAAC94, our product candidate to treat arthritis, and the patient subsequently died. In their review of the SAE, both the National Institutes of Health Recombinant DNA Advisory Committee and the trial s independent data safety monitoring board concluded that the patient s death was caused by complications from an opportunistic infection, not by our tgAAC94 product candidate, as described in our Current Report on Form 8-K filed on December 6, 2007. In addition, after the U.S. Food and Drug Administration, or FDA, reviewed the safety data on all 127 patients in the trial and data from the SAE, the FDA removed the hold it originally put on the clinical trial, permitting the clinical trial to resume. On March 3, 2009, we were served with a lawsuit filed by the patient s spouse, Robbie Mohr. The lawsuit was filed on August 18, 2008 in the 4th Judicial Circuit of Christian County, Illinois, against us, Abbot [sic] Laboratories Inc., and Western Institutional Review Board Inc. The complaint for the lawsuit alleges that the named parties negligence was the proximate cause of the patient s death and seeks unspecified compensatory damages in excess of $50,000.

Item 1A. Risk Factors.

In addition to the other information contained in this annual report, you should carefully read and consider the following risk factors. If any of these risks actually occur, our business, operating results or financial condition could be harmed. This could cause the trading price of our stock to decline, and you could lose all or part of your investment.

Risks Related to Our Business

If we are unable to raise additional capital or secure additional sources of funding in the very near term, we will be unable to continue our operations.

We have very limited capital resources and continue to incur significant operating losses, which threaten, and raise substantial doubt about, our ability to continue as a going concern. We currently expect that our existing financial resources will be sufficient to fund our operations only until the end of the second quarter of 2009, and actual results could differ from this estimate. This estimate is based on our ability to successfully perform planned activities and the receipt of expected funding under our collaborations and grants, and actual results could differ from our estimates. If we are unable to secure additional capital by the end of the second quarter of 2009, we expect to begin the process of ceasing operations, seeking bankruptcy protection or otherwise winding up our business. Given the short amount of time and the fact that we have so far been unable to secure additional funding sources notwithstanding our considerable efforts to date, we believe it is increasingly unlikely we will be able to secure additional financial resources in time. Even if we are able to extend our cash horizon, if we do not receive sufficient additional funding before we reach the end of our financial resources, we will nevertheless be forced to cease operations, seek bankruptcy protection or otherwise wind up our business.

The report of our independent registered public accounting firm on our audited financial statements included in our annual report on Form 10-K for the fiscal year ended December 31, 2008 contains a statement noting that we have incurred recurring losses and negative cash flows from operations that, due to our limited working capital, raise substantial doubt about our ability to continue as a going concern. Our plans to address these issues, which are discussed elsewhere in this report, are subject to numerous risks and contingencies, many of which are beyond our control, and we can give no assurance as to whether or how long we may be able to maintain our viability as a going concern.

Because our internally generated cash flow will not fund development and commercialization of our product candidates, even if we are able to secure additional financial resources in time to continue our operations, which we believe is increasingly unlikely, we will require substantial additional financial resources to continue to conduct business. Our short-term and long-term future capital requirements will depend on many factors. In the short term, our capital requirements depend on factors such as:

- whether we decide to continue to pursue all or a portion of our current research and development programs, including continuing to secure and protect intellectual property related to these programs;

the number of employees required to maintain our product development and manufacturing operations and also provide appropriate levels of general and administrative support;

our success in performing under our agreements with Celladon Corporation and the grant from the Department of Defense, or DOD, for our amyotrophic lateral sclerosis, or ALS, program, and in conducting the remaining wind-down activities and collecting remaining milestone payments under the vaccine program funded by the National Institute of Allergy and Infectious Disease, or NIAID; and

Table of Contents

the availability and success of collaborative, licensing, manufacturing or other agreements with or grants by third parties, and receiving payments under such agreements or grants when and as we anticipate.

In the longer term, our future capital requirements will depend on a number of factors, including:

whether we decide to pursue all or a portion of our current or future research and development programs;

the timing, costs and scope of, and our success in, conducting clinical trials, obtaining regulatory approvals and maintaining and expanding our patent portfolio;

the availability and success of collaborative, licensing, manufacturing or other agreements with third parties, and receiving payments under such agreements or grants when and as we anticipate;

our success in pursuing a settlement of our remaining obligations under our facility lease in Bothell, Washington after surrendering control of that facility in February 2009 and discontinuing rent payments, and whether the landlord terminates the lease as a result of this default and, among other potential remedies, accelerates our obligations due under the lease;

the rate and extent of scientific progress in our research and development programs;

whether we are successful in transferring our manufacturing technology and know-how to a contract manufacturing organization, or CMO;

competing technological and market developments;

which intellectual property we secure and protect related to our and our collaborators research and development programs;

the existence and outcome of any litigation or administrative proceedings, including those involving intellectual property; and

the timing and costs of, and our success in, any product commercialization activities and facility expansions, if and as required.

Additional sources of financing could involve one or more of the following:

strategic transactions, such as mergers and acquisitions;

selling or licensing our technology or product candidates;

extending or expanding our current product development or manufacturing collaborations, or entering into additional product development or manufacturing collaborations;

issuing equity or debt in the public or private markets; and/or

borrowing under loan or equipment financing arrangements.

Additional funding may not be available to us on reasonable terms, if at all. The capital markets have been experiencing extreme volatility and disruption for over a year, and the volatility and disruption have reached unprecedented levels in recent months. The scope and extent of this disruption in the capital markets could make it difficult or impossible to raise additional capital in public or private capital markets until conditions stabilize, and conditions may not stabilize in the very short amount of time left before we reach the end of our financial resources and we are forced to cease operations, seek bankruptcy protection or otherwise wind up our business.

If we raise additional funds through the issuance of equity or debt securities, the securities may have rights, preferences or privileges senior to those of the rights of our common stock, and our common stockholders will experience additional dilution. The perceived risk associated with the possible sale of a large number of shares of our common stock could cause some of our shareholders to sell their stock, thus causing the price of our stock to decline. In addition, actual or anticipated downward

Table of Contents

pressure on our stock price due to actual or anticipated sales of stock could cause some institutions or individuals to engage in short sales of our common stock, which may itself cause the price of our stock to decline.

If our stock price continues to decline, or does not increase sufficiently, we may be unable to raise additional capital. Additional declines in the price of our common stock, or a failure of the price of our common stock to increase sufficiently, could also impair our ability to attract and retain qualified employees, reduce the liquidity of our common stock and result in the delisting of our common stock from the NASDAQ Capital Market. Even if our stock price increases sufficiently, we nonetheless expect to be delisted because of non-compliance with the $2.5 million shareholders equity requirement of Marketplace Rule 4310(c)(3) resulting from our fourth quarter 2008 goodwill impairment and our $7.6 million of accrued Bothell facility obligations recorded in accordance with Statement of Financial Accounting Standards No. 146. If we are delisted from the NASDAQ Capital Market as we currently expect, then our ability to raise additional capital through the equity markets will be substantially harmed. Debt financing, if available, may require that we pledge our assets, including our intellectual property, or may require restrictive covenants that would restrict our business activities.

The funding that we expect to receive from our collaborations depends on continued scientific progress under the collaborations and our collaborators ability and willingness to continue or extend or fund the collaboration. If we are unable to successfully access sufficient additional capital, we may need to scale back, delay or terminate one or more of our development programs, suspend prosecution of our intellectual property portfolio, or reduce other operating activities or workforce, which could result in a loss or reduction of funding under any affected collaboration. We may also be required to sell or relinquish some rights to our technology or product candidates or grant or take licenses on unfavorable terms, either of which would reduce the ultimate value to us of our technology or product candidates.

We expect to continue to operate at a loss and may never become profitable.

Substantially all of our revenue since 2005 has been derived from collaborative research and development agreements in connection with the development of our potential product candidates, including our collaborations with Celladon and the International AIDS Vaccine Initiative, or IAVI, and our subcontract with Nationwide Children s Hospital, or NCH, and Children s Hospital of Philadelphia, or CHOP, funded by the NIAID. We have incurred, and will continue to incur for the foreseeable future, significant expense to develop our research and development programs, conduct preclinical studies and clinical trials, seek regulatory approval for our product candidates and provide general and administrative support for these activities. As a result, we have incurred significant net losses since inception, and we expect to continue to incur substantial additional losses in the future.

As of March 31, 2009, we had an accumulated deficit of $322.7 million. We may never be able to commercialize our products or generate profits and, if we do become profitable, we may be unable to sustain or increase profitability.

All of our product candidates are in preclinical development or early-stage clinical trials, and if we and our partners are unable to successfully develop, commercialize and market our product candidates, we will be unable to generate sufficient capital to maintain our business.

As of March 31, 2009, the heart failure product candidate developed under our collaboration with Celladon is in a Phase I/II clinical trial, the HIV/AIDS product candidate developed under our collaboration with IAVI has completed both a Phase I and Phase II trial, the product candidate for Leber s congenital amaurosis, or LCA, developed under our collaboration with the University College London/Moorfields Eye Hospital is in a Phase I/II clinical trial, we have completed a Phase I/II trial of our inflammatory arthritis candidate, and we have no product candidates in Phase III trials. Our product candidates for ALS and Huntington s disease, or HD, are currently in preclinical development, and based on anticipated product development timelines and our current financial resources, we are evaluating our continued involvement in these programs. Of the product candidates that we and/or our partners are currently developing, we will not generate any product revenue, commercial manufacturing revenue, revenue sharing or royalties for at least several years, and then only if we and/or our partners can successfully commercialize our product candidates. Commercializing our potential products depends on successful completion of additional research and development and testing, in both preclinical development and clinical trials. Clinical trials may take several years or more to complete. The commencement, cost and rate of completion of our clinical trials may vary or be delayed for many reasons. If we are unable to successfully complete preclinical and clinical development of some or all of our product candidates in a timely manner, we may be unable to generate sufficient product revenue to maintain our business.

Even if our potential products succeed in clinical trials and are approved for marketing, these products may never achieve market acceptance. If we are unsuccessful in marketing or commercializing our product candidates for any reason, including greater effectiveness or economic feasibility of competing products or treatments, the failure of the medical community or the

15

Table of Contents

public to accept or use any products based on gene delivery, inadequate marketing and distribution capabilities or other reasons discussed elsewhere in this section, we will be unable to generate sufficient product revenue to maintain our business.

Our recent reductions in force may harm our business.

In order to decrease our ongoing cost structure, we have decreased our headcount through voluntary and involuntary employee terminations in all areas of our business. Our employee headcount decreased from 68 full-time equivalent employees at September 30, 2008 to approximately 35 full-time equivalent employees at May 1, 2009. These staff reductions may impact our ability to execute on our business strategy and may result in failure to accomplish our business objectives. For example, recent headcount reductions in our product development staff could impair our ability to enter into new product research and development agreements and delay or hinder our performance under such agreements, and if the anticipated negative effects of headcount reductions on our product development programs are greater than or different than we anticipated, our ability to successfully develop our current product candidates could be harmed. In addition, our reductions in force may yield unanticipated consequences, such as attrition beyond our planned reductions, and we may encounter difficulty in managing our business as a result.

If we do not retain our existing personnel and attract and retain qualified personnel in the future, we may be unable to manage our business and develop and commercialize some of our potential products.

Our future success depends in large part on the efforts and abilities of, and our ability to attract and retain, key technical and management personnel. All of our employees, including our executive officers, can terminate their employment with us at any time. We have programs in place designed to retain personnel, including competitive compensation packages and programs to create a positive work environment. Other companies, research and academic institutions and other organizations in our field compete intensely for employees, however, and we may be unable to retain our existing personnel or, when and if we obtain additional funding for our operations, hire additional qualified employees and consultants. We instituted several reductions in force in 2008 and 2009, our chief executive officer and chief scientific officer resigned in November 2008 and we are, and for about a year have been, operating with a very short cash horizon. This may give rise to uncertainty, which may make it more difficult to retain our current personnel and attract and retain qualified personnel in the future. In addition, our ability to attract and retain qualified employees may be adversely affected if the price of our common stock fails to increase sufficiently or declines in the future or if, as we currently expect, our stock is delisted from the NASDAQ Capital Market. If we experience significant turnover or difficulty in recruiting new personnel, our ability to manage our business could be impaired, our research and development of product candidates could be delayed and we could experience difficulty in generating sufficient cash funding to maintain our business.

If we lose our collaborative partners or we do not receive the funding we anticipate under our collaborative agreements or grants, we may be unable to develop our potential products.

A substantial portion of our operating expenses are funded through our collaborative agreements with third parties. We have a heart failure program funded by a biotechnology company, Celladon, and a grant from the U.S. Department of Defense, or DOD, in support of developing a product candidate to treat ALS. Our HIV/AIDS vaccine collaboration with CHOP and NCH is funded through a subcontract with NIAID, which is a U.S. government agency. Each of these collaborations or grants provides for funding, collaborative development, intellectual property rights and/or expertise to develop certain of our product candidates. The Celladon contract providing funding for our development and manufacturing efforts terminates at the end of that campaign, but no later than July 31, 2009. We also expect to complete the work plan for the DOD-funded preclinical efforts for ALS by the end of the fourth quarter of 2009. We expect to complete our development and manufacturing work related to the NIAID-funded HIV/AIDS vaccine candidate in the first half of 2009 and terminate our involvement in this program, as the vaccine candidate enters into clinical testing. To the extent that we lose collaborative partners or grant funding for a program or a portion of a program that we do not fund internally, or to the extent that we do not receive the funding that we expect from our collaborative agreements or grants, unless we are able to obtain alternative sources of funding, we would be delayed in or unable to continue developing potential products under the affected program (or, in the case of Celladon, complete the current manufacturing campaign). With limited exceptions, each collaborator or grantor has the right to terminate its obligation to provide research funding at any time for scientific or business reasons. For example, in 2008 Sirna Therapeutics, a wholly-owned subsidiary of Merck & Co., Inc., ceased collaborating with us on our HD program and instead transferred the rights necessary to conduct the program to us. In addition, to the extent that funding is provided by a collaborator for non-program-specific uses, the loss of significant amounts of collaborative funding could result in the delay, reduction or termination of additional research and development programs, a reduction in capital expenditures or business development and other operating activities, or any combination of these measures, which could seriously harm our business.

Table of Contents

We may not be able to obtain and maintain the additional third-party relationships that are necessary to develop, commercialize and manufacture some or all of our product candidates or to expand our pipeline by adding new candidates.

We expect to depend on collaborators, partners, licensees, contract research organizations, or CROs, manufacturers and other third parties and strategic partners to support and fund our discovery and development efforts, to formulate product candidates, to conduct clinical trials for some or all of our product candidates, to manufacture clinical and commercial scale quantities of our product candidates and products and to market, sell, and distribute any products we successfully develop. We cannot guarantee that we will be able to successfully negotiate agreements for or maintain relationships with additional collaborators, partners, licensees, clinical investigators, manufacturers and other third parties on favorable terms, if at all. If we are unable to obtain or maintain these agreements, we may not be able to clinically develop, formulate, manufacture, obtain regulatory approvals for or commercialize our product candidates, which will in turn adversely affect our business. For example, we do not intend to move the development of our treatment of inflammatory arthritis, tgAAC94, forward to additional clinical studies without additional external funding from a third party.

We expect to expend substantial management time and effort to enter into relationships with third parties and, if we successfully enter into such relationships, to manage these relationships. In addition, substantial amounts of our expenditures will be paid to third parties in these relationships. However, we cannot control the amount or timing of resources our contract partners will devote to our research and development programs, product candidates or potential product candidates, and we cannot guarantee that these parties will fulfill their obligations to us under these arrangements in a timely fashion, if at all.

If our clinical trials are delayed, suspended or terminated, we may be unable to develop our product candidates on a timely basis, which could increase our development costs, delay the potential commercialization of our products, and make it difficult to raise additional capital.

We cannot predict whether we will encounter problems with any of our completed, ongoing or planned clinical trials that will cause regulatory agencies, institutional review boards or us to delay our clinical trials or suspend or delay the analysis of the data from those trials. Clinical trials can be delayed for a variety of reasons, including:

- the placement of a clinical hold on a trial, such as the four-month clinical hold placed on our Phase I/II clinical trial of tgAAC94, our inflammatory arthritis product candidate, in 2007 after a patient participating in the clinical trial experienced a serious adverse event, or SAE, and subsequently died;
- the occurrence of drug-related side effects or adverse events experienced by participants in our clinical trials;
- discussions with the U.S. Food and Drug Administration, or FDA, or comparable foreign authorities regarding the scope or design of our clinical trials;
- delays or the inability to obtain required approvals from institutional review boards or other governing entities at clinical sites selected for participation in our clinical trials;
- delays in enrolling patients into clinical trials;
- lower than anticipated retention rates of patients in clinical trials;
- the need to repeat or conduct additional clinical trials as a result of problems such as inconclusive or negative results, poorly executed testing or unacceptable design;

an insufficient supply of product candidate materials or other materials necessary to conduct our clinical trials;

the need to qualify new suppliers of product candidate materials for FDA and foreign regulatory approval; or

an unfavorable FDA inspection or review of a clinical trial site or records of any clinical investigation.

Table of Contents

If our clinical trials are delayed or terminated, we may be unable to develop our product candidates on a timely basis, which may increase our development costs and could delay the potential commercialization of our products and the subsequent receipt of revenue from sales, if any.

In addition, a clinical trial may be suspended or terminated by us, the FDA or other regulatory authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- inspection of the clinical trial operations or trial sites by the FDA or other regulatory authorities resulting in the imposition of a clinical hold;
- unforeseen safety issues or any determination that a trial presents unacceptable health risks; or
- lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional trials and studies and increased expenses associated with the services of our CROs and other third parties.

Changes in regulatory requirements and guidance may occur and we may need to amend clinical trial protocols to reflect these changes. Amendments may require us to resubmit our clinical trial protocols to institutional review boards for reexamination, which may impact the costs, timing or successful completion of a clinical trial. If the results of our clinical trials are not available when we expect or if we encounter any delay in the analysis of data from our clinical trials, we may be unable to file for regulatory approval or conduct additional clinical trials on the schedule we currently anticipate. Any delays in completing our clinical trials may increase our development costs, slow down our product development and approval process, delay our receipt of product revenue and make it difficult to raise additional capital. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of a product candidate, which would seriously harm our business. In addition, significant clinical trial delays also could allow our competitors to bring products to market before we do and impair our ability to commercialize our future products and may seriously harm our business.

Litigation involving intellectual property, product liability or other claims and product recalls could strain our resources, subject us to significant liability, damage our reputation or result in the invalidation of our proprietary rights.

As our product development efforts progress, most particularly in potentially significant markets such as HIV/AIDS, heart failure or ALS therapies, the risk increases that others may claim that our processes and product candidates infringe on their intellectual property rights. In addition, administrative proceedings, litigation or both may be necessary to enforce our intellectual property rights or determine the rights of others. Defending or pursuing these claims, regardless of their merit, would be costly and would likely divert management s attention and resources away from our operations. If there were to be an adverse outcome in litigation or an interference proceeding, we could face potential liability for significant damages or be required to obtain a license to the patented process or technology at issue, or both. If we are unable to obtain a license on acceptable terms, or to develop or obtain alternative technology or processes, we may be unable to manufacture or market any product or potential product that uses the affected process or technology.

Clinical trials and the marketing of any potential products may expose us to liability claims resulting from the testing or use of our products. Gene therapy treatments are new and unproven, and potential known and unknown side effects of gene therapy may be serious and potentially life-threatening. Product liability claims may be made by clinical trial participants, consumers, healthcare providers or other sellers or users of our products. For example, a patient in one of our clinical trials experienced an SAE and subsequently died. Even though the NIH s Office of Biotechnology Recombinant DNA Advisory Committee, or RAC, and the trial s independent data safety monitoring board determined that the SAE was not caused by our drug, the spouse of that patient has filed a lawsuit alleging that various named parties negligence, including ours, was the proximate cause of the patient s death. Although we currently maintain liability insurance, the costs of product liability and other claims against us may exceed our insurance coverage. In addition, we may require increased liability coverage as additional product candidates are used in clinical trials or commercialized. Liability insurance is expensive and may not continue to be available on acceptable terms. A product liability or other claim or product recall not covered by or exceeding our insurance coverage could significantly harm our financial condition. In addition, adverse publicity resulting from a product recall or a liability claim against us, one of our partners or another gene therapy company could significantly harm our reputation and make it more difficult to obtain the funding and collaborative partnerships necessary to maintain our business.

18

Table of Contents

Failure to recruit subjects could delay or prevent clinical trials of our potential products, which could delay or prevent the development of potential products.

Identifying and qualifying subjects to participate in clinical trials of our potential products is critically important to our success. The timing of our clinical trials depends on the speed at which we can recruit subjects to participate in testing our product candidates. We have experienced delays in some of our clinical trials, and we may experience similar delays in the future. If subjects are unwilling to participate in our gene therapy trials because of negative publicity from or concerns about the death of a subject in one of our trials who suffered an SAE, or adverse events in the biotechnology or gene therapy industries in general or for other reasons, including competitive clinical trials for similar patient populations, the timeline for recruiting subjects, conducting trials and obtaining regulatory approval of potential products will be delayed. 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 which could seriously harm our business.

Because our product candidates involve new and unproven technologies, the regulatory approval process may proceed more slowly compared to clinical trials involving new candidates in already proven drug classes.

No gene therapy products have received regulatory approval for marketing from the FDA. Because our product candidates involve new and unproven technologies, we believe that the regulatory approval process may proceed more slowly compared to clinical trials involving new candidates in already proven drug classes. The FDA and applicable state and foreign regulators must conclude at each stage of clinical testing that our clinical data suggest acceptable levels of safety in order for us to proceed to the next stage of clinical trials. In addition, gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the National Institutes of Health, or NIH, are subject to review by the RAC. Although the RAC does not have regulatory status, the RAC review process can impede the initiation of the trial, because no research participant can be enrolled until the RAC review process has been completed and Institutional Biosafety Committee approval (from the clinical trial site) has been obtained, even if the FDA has reviewed and approved the protocol and initiation of clinical trial.

The regulatory approval process for our product candidates is costly, time-consuming and subject to unpredictable changes and delays, and our product candidates may never receive regulatory approval or be found safe and effective.

Both before and after approval of our product candidates, we, our product candidates and our suppliers are subject to extensive regulation by governmental authorities in the United States and other countries, covering, among other things, testing, manufacturing, quality control, labeling, advertising, promotion, distribution, and import and export. Failure to comply with applicable requirements could result in, among other things, one or more of the following actions: warning letters; fines and other monetary penalties; unanticipated expenditures; delays in approval or refusal to approve a product candidate; product recall or seizure; interruption of manufacturing or clinical trials; operating restrictions; injunctions; and criminal prosecution. We or the FDA may suspend or terminate human clinical trials at any time on various grounds. For example, after an SAE occurred in our 2007 Phase I/II clinical trial of tgAAC94, our inflammatory arthritis product candidate, the FDA placed a hold on the trial for several months in order to conduct in-depth review of data. Although the SAE was determined to be unrelated to our product, completion of the trial was delayed by approximately six months because of the hold.

All of our product candidates are in development, and will have to be approved by the FDA before they can be marketed in the United States. The FDA has not approved any of our product candidates for sale in the United States and no company has sought FDA approval of a gene therapy based product. The clinical trial requirements of the FDA and other regulatory agencies 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 of the potential products. In addition, regulatory requirements governing gene therapy products have changed frequently and may change in the future. Obtaining FDA approval requires substantial time, effort, and financial resources, and may be subject to both expected and unforeseen delays, and we can provide no assurance that any approval will be granted on a timely basis, if at all.

The FDA may decide that our data are insufficient for approval of our product candidates and may require additional preclinical, clinical or other studies. As we develop our product candidates, we periodically discuss with the FDA clinical, regulatory and manufacturing matters, and our views may, at times, differ from those of the FDA.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate for regulatory approval, if we are unable to successfully complete our clinical trials or other testing, or if the results of these and other trials or tests fail to demonstrate efficacy or raise safety concerns, we may be delayed in obtaining marketing approval for our product candidates, or may never be able to obtain marketing approval. Should this occur, we may have to delay or discontinue development of the product candidate, and the partner, if any, that supports development of that product candidate may terminate its support. Even a product candidate that appears promising at an early stage of research or development may not

19

Table of Contents

result in a commercially successful product. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market will decrease our ability to generate sufficient product revenue to maintain our business.

Even if regulatory approval of a product candidate is obtained, such approval may be subject to significant limitations on the indicated uses for which that product may be marketed, conditions of use, and/or significant post approval obligations, including additional clinical trials. These regulatory requirements may, among other things, limit the size of the market for the product. Even after approval, discovery of previously unknown problems with a product, manufacturer or facility, such as previously undiscovered side effects, may result in restrictions on any product, manufacturer or facility, including, among other things, a possible withdrawal of approval of the product, which would seriously harm our business.

If we are unable to obtain or maintain licenses for necessary third-party technology on acceptable terms or to develop alternative technology, we may be unable to develop and commercialize our product candidates.

We have entered into exclusive and nonexclusive license agreements that give us and our partners rights to use technologies owned or licensed by commercial and academic organizations in the research, development and commercialization of our potential products. We believe that we will need to obtain additional licenses to use patents and unpatented technology owned or licensed by others for use, compositions, methods, processes to manufacture compositions, processes to manufacture and purify gene therapeutics candidates and other technologies and processes for our present and potential product candidates. If we are unable to maintain our current licenses for third-party technology or obtain additional licenses on acceptable terms, 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. In addition, the license agreements for technology for which we hold exclusive licenses typically contain provisions that require us to meet minimum development milestones in order to maintain the license on an exclusive basis for some or all fields of the license. We also have license agreements for some of our technologies that may require us to sublicense certain of our rights. If we do not meet these requirements, our licensor may convert all or a portion of the license to a nonexclusive license or, in some cases, terminate the license.

In many cases, patent prosecution of our licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- 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; and
- the priority of invention of patented technology.

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, which could seriously harm our business.

If our partners or scientific consultants terminate, reduce or delay our relationships with them, we may be unable to develop our potential products.

Our partners provide funding, manage regulatory filings, aid and augment our internal research and development efforts and provide access to important intellectual property and know-how. Their activities include, for example, support in processing the regulatory filings of our product candidates and funding clinical trials. Our outside scientific consultants and contractors perform research, develop technology and processes to advance and augment our internal efforts and provide access to important intellectual property and know-how. Their activities may include, for example, clinical evaluation of our product candidates, product development activities performed under our research collaborations, research under sponsored research agreements and

Table of Contents

certain contract manufacturing-related services. Collaborations with established pharmaceutical and biotechnology companies and academic, research and public health organizations often provide a measure of validation of our product development efforts in the eyes of securities analysts, investors and the medical community. The development of certain of our potential products, and therefore the success of our business, depends on the performance of our partners, consultants and contractors. If they do not dedicate sufficient time, regulatory or other technical resources to the research and development programs for our product candidates or if they do not perform their obligations as expected, we may experience delays in, and may be unable to continue, the preclinical or clinical development of those product candidates. Each of our collaborations and scientific consulting relationships concludes at the end of the term specified in the applicable agreement unless we and our partners agree to extend the relationship. Any of our partners may decline to extend the collaboration, or may be willing to extend the collaboration only with a significantly reduced scope. Competition for scientific consultants and partners in gene therapy is intense. We may be unable to successfully maintain our existing relationships or establish additional relationships necessary for the development of our product candidates on acceptable terms, if at all. If we are unable to do so, our research and development programs may be delayed or we may lose access to important intellectual property or know-how.

We rely on third parties to conduct our preclinical research and clinical trials. If these third parties do not perform as contractually required or otherwise expected, we may not be able to obtain regulatory approval for or commercialize our product candidates.

We rely on third parties, such as CROs and research institutions, to conduct a portion of our preclinical research. We also rely on third parties, such as medical institutions, clinical investigators and CROs, to assist us in conducting our clinical trials. Nonetheless, we are responsible for confirming that our preclinical research is conducted in accordance with applicable regulations, and that our clinical trials are conducted in accordance with applicable regulations, the relevant protocol and within the context of approvals by an institutional review board. Our reliance on these third parties does not relieve us of responsibility for ensuring compliance with FDA regulations and standards for conducting, monitoring, recording and reporting the results of preclinical research and clinical trials to ensure that data and reported results are credible and accurate and that the trial participants are adequately protected. If these third parties do not successfully carry out their contractual duties or regulatory obligations or meet expected deadlines, if the third parties need to be replaced or if the quality or accuracy of the data they obtain is compromised due to their failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our preclinical and clinical development processes may be extended, delayed, suspended or terminated, and we may not be able to obtain regulatory approval for our product candidates.

Any success of our clinical trials and preclinical studies may not be indicative of results in a large number of subjects of either safety or efficacy.

The successful results of our technology in preclinical studies using animal models may not be predictive of the results that we will see in our clinical trials with human subjects. In addition, results in early-stage clinical trials generally test for drug safety rather than efficacy and are based on limited numbers of subjects. Drug development involves a high degree of risk and our reported progress and results from our early phases of clinical testing of our product candidates may not be indicative of progress or results that will be achieved from larger populations, which could be less favorable. Moreover, we do not know if any favorable results we achieve in clinical trials will have a lasting or repeatable effect. If a larger group of subjects does not experience positive results or if any favorable results do not demonstrate a beneficial effect, our product candidates that we advance to clinical trials may not receive approval from the FDA for further clinical trials or commercialization. For example, in March 2005, we discontinued the development of tgAAVCF, our product candidate for the treatment of cystic fibrosis, following the analysis of Phase II clinical trial data in which tgAAVCF failed to achieve the efficacy endpoints of the trial.

We may be unable to adequately protect our proprietary rights domestically or overseas, which may limit our ability to successfully market any product candidates.

Our success depends substantially on our ability to protect our proprietary rights and operate without infringing on the proprietary rights of others. We own or license patents and patent applications and will need to license additional patents for genes, processes, practices and techniques critical to our present and potential product candidates. If we fail to obtain and maintain patent or other intellectual property protection for this technology, our competitors could market competing products using those genes, processes, practices and techniques. The patent process takes several years and involves considerable effort and expense. In addition, patent applications and patent positions in the field of biotechnology are highly uncertain and involve complex legal, scientific and factual questions. Our patent applications may not result in issued patents and the scope of any patent may be reduced both before and after the patent is issued. Even if we secure a patent, the patent may not provide significant protection and may be circumvented or invalidated.

Table of Contents

We also rely on unpatented proprietary technology and technology that we have licensed on a nonexclusive basis. While we take precautions to protect our proprietary unpatented technology, we may be unable to meaningfully protect this technology from unauthorized use or misappropriation by a third party. Our competitors could also obtain rights to our nonexclusively licensed proprietary technology. In any event, other companies may independently develop equivalent proprietary information and techniques. If our competitors develop and market competing products using our unpatented or nonexclusively licensed proprietary technology or substantially similar technology, our products, if successfully developed, could suffer a reduction in sales or be forced out of the market.

In 2008 and continuing into 2009, we reviewed our very broad-based AAV patent portfolio. We determined that, based on the status of our and others current product development efforts and our current financial resources, certain intellectual property assets are not essential to our current business strategy and we have therefore either returned those rights to our licensors or ceased prosecution of those patents. Although we do not believe those proprietary rights are essential to our current business strategy, the loss of those rights could limit our business opportunities, including our ability to enter into strategic transactions such as mergers and acquisitions, license our technology, sell our product development programs or products, if successfully developed, or raise capital through issuing equity or debt.

If we do not develop adequate development, manufacturing, sales, marketing and distribution capabilities, either alone or with our business partners, we will be unable to generate sufficient product revenue to maintain our business.

Our potential products require significant development of new processes and design for the advancement of the product candidate through manufacture, preclinical and clinical testing. We may be unable to continue development or meet critical milestones with our partners due to technical or scientific issues related to manufacturing or development. We currently do not have the physical capacity to manufacture large-scale quantities of our potential products. This could limit our ability to conduct large clinical trials of a product candidate and to commercially launch a successful product candidate. In order to manufacture product at such scale, we will need to expand or improve our current facilities and staff or supplement them through the use of contract providers. For example in February 2009 we and Celladon agreed to transfer the manufacture of Celladon s MYDICAR® product to an external contract manufacturing organization. If we are unable to obtain and maintain the necessary manufacturing capabilities, either alone or through third parties, we will be unable to manufacture our potential products in quantities sufficient to sustain our business or achieve profitability. Moreover, we are unlikely to become profitable if we, or our contract providers, are unable to manufacture our potential products in a cost-effective manner.

In addition, we have no experience in sales, marketing and distribution. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. We intend to enter into collaborations with other entities to utilize their mature marketing and distribution capabilities, but we may be unable to enter into marketing and distribution agreements on favorable terms, if at all. If our current or future collaborative partners do not commit sufficient resources to timely marketing and distributing our future products, if any, and we are unable to develop the necessary marketing and distribution capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business.

Our product candidates may never achieve market acceptance even if we obtain regulatory approvals.

Even if we obtain regulatory approvals for the commercial sale of our product candidates, the commercial success of these product candidates will depend on, among other things, their acceptance by physicians, patients, third-party payors and other members of the medical community as a therapeutic and cost-effective alternative to competing products and treatments. If our product candidates fail to gain market acceptance, we may be unable to earn sufficient revenue to continue our business. Market acceptance of, and demand for, any product that we may develop and commercialize will depend on many factors, including:

- the prevalence of adverse side effects;
- availability, relative cost, and relative efficacy of alternative and competing treatments;
- the effectiveness of our marketing and distribution strategy;
- publicity concerning our products or competing products and treatments; and

our ability to obtain sufficient third-party insurance coverage or reimbursement.

If our product candidates do not become widely accepted by physicians, patients, third-party payors, and other members of the medical community, we would be unable to generate sufficient revenue to sustain our business.

Table of Contents

Post-approval manufacturing or product problems or failure to satisfy applicable regulatory requirements could prevent or limit our ability to market our products.

Commercialization of any products will require continued compliance with the FDA and other federal, state and local regulations. For example, our current manufacturing facility, which is designed for manufacturing our adeno-associated virus, or AAV, vectors for clinical and development purposes, is subject to the Good Manufacturing Practices requirements and other regulations of the FDA, as well as to other federal, state and local regulations such as the Occupational Health and Safety Act, the Toxic Substances Control Act, the Resource Conservation and Recovery Act and the Environmental Protection Act. Any future manufacturing facility that we may construct for large-scale commercial production will also be subject to regulation. We may be unable to obtain regulatory approval for or maintain in operation this or any other manufacturing facility. In addition, we may be unable to attain or maintain compliance with current or future regulations relating to manufacture, safety, handling, storage, record keeping or marketing of potential products. If we fail to comply with applicable regulatory requirements or discover previously unknown manufacturing, contamination, product side effects or other problems after we receive regulatory approval for a potential product, we may suffer restrictions on our ability to market the product or be required to withdraw the product from the market.

We rely on single third-party suppliers for some of our raw materials; if these third parties fail to supply these items, development of affected product candidates may be delayed or discontinued.

Certain raw materials necessary for the manufacturing and formulation of our product candidates are provided by single-source unaffiliated third-party suppliers. We would be unable to obtain these raw materials for an indeterminate period of time if these third-party single-source suppliers were to cease or interrupt production or otherwise fail to supply these materials to us for any reason, including:

regulatory requirements or action by the FDA or others;

adverse financial developments at or affecting the supplier;

unexpected demand for or shortage of raw materials;

labor disputes or shortages; and

failure to comply with our quality standards, which results in quality failures, product contamination and/or recall.

For example, we have experienced issues in the past with obtaining certain raw materials we use for vector production due to quality problems at the suppliers. These events could adversely affect our ability to continue development on affected product candidates, which could seriously harm our business.

Risks Related to Our Industry

Adverse events in the field of gene therapy could damage public perception of our potential products and negatively affect governmental approval and regulation.

Public perception of our product candidates could be harmed by negative events in the field of gene transfer. For example, in 2003, 14 subjects in a French academic clinical trial being treated for x-linked severe combined immunodeficiency in a gene therapy trial using a retroviral vector showed correction of the disease, although three of the subjects subsequently developed leukemia. A subject in one of our trials died in 2007 after suffering an SAE that ultimately was attributed to an opportunistic infection. Adverse events in our clinical trials, such as happened in 2007, even if not ultimately attributable to our drug candidates, and the resulting publicity, as well as any other adverse events in the field of gene therapy that may occur in the future, could result in a decrease in demand for any products that we may develop. The commercial success of our product candidates will depend in part on public acceptance of the use of gene therapy for preventing or treating human diseases. If public perception is influenced by claims that gene therapy is unsafe, our product candidates may not be accepted by the general public or the medical community, which may conclude that our technology is unsafe.

Future adverse events in gene therapy or the biotechnology industry could also result in greater governmental regulation, unfavorable public perception, stricter labeling requirements and potential regulatory delays in the testing or approval of our potential products. Any increased scrutiny could delay or increase the costs of our product development efforts or clinical trials.

Table of Contents

Our use of hazardous materials exposes us to liability risks and regulatory limitations on their use, either of which could reduce our ability to generate product revenue.

Our research and development activities involve the controlled use of hazardous materials, including chemicals, biological materials and radioactive compounds. Our safety procedures for handling, storing and disposing of these materials must comply with federal, state and local laws and regulations, including, among others, those relating to solid and hazardous waste management, biohazard material handling, radiation and air pollution control. We may be required to incur significant costs in the future to comply with environmental or other applicable laws and regulations. In addition, we cannot eliminate the risk of accidental contamination or injury from hazardous materials. If a hazardous material accident were to occur, we could be held liable for any resulting damages, and this liability could exceed our insurance and financial resources. Accidents unrelated to our operations could cause federal, state or local regulatory agencies to restrict our access to hazardous materials needed in our research and development efforts, which could result in delays in our research and development programs. Paying damages or experiencing delays caused by restricted access could reduce our ability to generate revenue and make it more difficult to fund our operations.

The intense competition and rapid technological change in our market may result in failure of our potential products to achieve market acceptance.

We face increasingly intense competition from a number of commercial entities and institutions that are developing gene therapy technologies. Our competitors include early-stage and more established gene delivery companies, other biotechnology companies, pharmaceutical companies, universities, research institutions and government agencies developing gene therapy products or other biotechnology-based therapies designed to treat the diseases on which we focus. We also face competition from companies using more traditional approaches to treating human diseases, such as surgery, medical devices and pharmaceutical products. If our product candidates become commercial gene therapy products, they may affect commercial markets of the analogous protein or traditional pharmaceutical therapy. This may result in lawsuits, demands, threats or patent challenges by others in an effort to reduce our ability to compete. In addition, we compete with other companies to acquire products or technology from research institutions or universities. Many of our competitors have substantially more resources, including research and development personnel, capital and infrastructure, than we do. Many of our competitors also have greater experience and capabilities than we do in:

- research and development;
- clinical trials;
- obtaining FDA and other regulatory approvals;
- manufacturing; and
- marketing and distribution.

In addition, the competitive positions of other companies, institutions and organizations, including smaller competitors, may be strengthened through collaborative relationships. Consequently, our competitors may be able to develop, obtain patent protection for, obtain regulatory approval for, or commercialize new products more rapidly than we do, or manufacture and market competitive products more successfully than we do. This could limit the prices we could charge for the products that we are able to market or result in our products failing to achieve market acceptance.

Gene therapy is a rapidly evolving field and is expected to continue to undergo significant and rapid technological change and competition. Rapid technological development by our competitors, including development of technologies, products or processes that are more effective or more economically feasible than those we have developed, could result in our actual and proposed technologies, products or processes losing market share or becoming obsolete.

Healthcare reform measures and the unwillingness of third-party payors to provide adequate reimbursement for the cost of our products could impair our ability to successfully commercialize our potential products and become profitable.

Sales of medical products and treatments, both domestically and abroad, substantially depend on the availability of reimbursement to the consumer from third-party payors. Our potential products may not be considered cost-effective by third-party payors, who may not provide coverage at the price set for our products, if at all. If purchasers or users of our products are unable to obtain adequate reimbursement, they may forego or reduce their use of our products. Even if coverage is provided, the

Table of Contents

approved reimbursement amount may not be high enough to allow us to establish or maintain pricing to realize a sufficient return on our investment.

Increasing efforts by governmental and third-party payors, such as Medicare, private insurance plans and managed care organizations, to cap or reduce healthcare costs will affect our ability to commercialize our product candidates and become profitable. We believe that third-party payors will attempt to reduce healthcare costs by limiting both coverage and level of reimbursement for new products approved by the FDA. There have been and will continue to be a number of federal and state proposals to implement government controls on pricing, the adoption of which could affect our ability to successfully commercialize our product candidates. Even if the government does not adopt any such proposals or reforms, their announcement could impair our ability to raise capital.

Risks Related to Our Common Stock

We expect that we will be unable to comply with the minimum requirements for quotation on the NASDAQ Capital Market and will be delisted from the NASDAQ Capital Market. As a result, we expect the liquidity and market price of our common stock to decline.

Our stock is listed on the NASDAQ Capital Market. In order to continue to be listed on the NASDAQ Capital Market, we must meet specific quantitative standards, including maintaining a minimum bid price of $1.00 for our common stock, a market value of $1.0 million for our publicly held shares (public float), and $2.5 million in shareholders equity. On April 23, 2008, we received a notice from the NASDAQ Stock Market, or NASDAQ, informing us that for 30 consecutive business days the bid price of our common stock had closed below the minimum $1.00 per share requirement for continued inclusion under Marketplace Rule 4310(c)(4). The letter stated that, under Marketplace Rule 4310(c)(8)(d), we would be provided with 180 calendar days to regain compliance with the bid price requirement. NASDAQ has since suspended enforcement of the bid price and public float requirements until July 20, 2009, at which time we will have five business days to regain compliance with those requirements. If, on July 27, 2009, we meet all of the NASDAQ Capital Market s initial listing criteria set forth in Marketplace Rule 4310(c) (other than the bid price criterion), but have not regained bid price compliance, we will be afforded an additional 180 calendar days to regain bid price compliance. To regain compliance with a listing standard, we must meet that standard for a minimum of 10 consecutive business days.

As a result of the goodwill impairment charge we recognized in the fourth quarter of 2008, combined with restructure charges totaling $7.6 million, our net worth has fallen to a deficit of $5.4 million at March 31, 2009, which is below the $2.5 million in shareholders equity required under Marketplace Rule 4310(c)(3). We do not meet the alternative continued listing requirements of $35.0 million in market value of listed securities or $500,000 of net income from continuing operations. On April 8, 2009, we received a letter from NASDAQ informing us of our non-compliance with Marketplace Rule 4310(c)(3). The letter stated that we had until April 23, 2009 to submit a plan to regain compliance and that, if the plan were to be accepted, we could be provided with up to 105 calendar days from April 8, 2009 to demonstrate compliance. On April 23, 2009, we submitted our compliance plan to NASDAQ.

If we were to be delisted from the NASDAQ Capital Market, trading, if any, in our shares may continue to be conducted on the Over-the-Counter Bulletin Board or in a non-NASDAQ over-the-counter market, such as the pink sheets. Delisting of our shares would result in limited release of the market price of those shares and limited analyst coverage and could restrict investors interest in our securities. Also, a delisting could have a material adverse effect on the trading market and prices for our shares and our ability to issue additional securities or to secure additional financing. In addition, if our shares were not listed and the trading price of our shares was less than $5.00 per share, our shares could be subject to Rule 15g-9 under the Securities Exchange Act of 1934, as amended, which, among other things, requires that broker/dealers satisfy special sales practice requirements, including making individualized written suitability determinations and receiving a purchaser s written consent prior to any transaction. In such case, our securities could also be deemed to be a penny stock under the Securities Enforcement and Penny Stock Reform Act of 1990, which would require additional disclosure in connection with trades in those shares, including the delivery of a disclosure schedule explaining the nature and risks of the penny stock market. Such requirements could severely limit the liquidity of our securities and our ability to raise additional capital in an already challenging capital market.

If we sell additional shares, our stock price may decline as a result of the dilution that will occur to existing shareholders.

Until we are profitable, we will need significant additional funds to develop our business and sustain our operations. Any additional sales of shares of our common stock are likely to have a dilutive effect on our then-existing shareholders. Subsequent sales of these shares in the open market could also have the effect of lowering our stock price, thereby increasing the number of shares we may need to issue in the future to raise the same dollar amount and consequently further diluting our outstanding

25

Table of Contents

shares. These future sales could also have an adverse effect on the market price of our shares and could result in additional dilution to the holders of our shares.

The perceived risk associated with the possible sale of a large number of shares could cause some of our shareholders to sell their stock, thus causing the price of our stock to decline. In addition, actual or anticipated downward pressure on our stock price due to actual or anticipated sales of stock could cause some institutions or individuals to engage in short sales of our common stock, which may itself cause the price of our stock to decline.

If our stock price declines or does not increase sufficiently, we may be unable to raise additional capital. As our existing financial resources are only expected to be sufficient to fund our operations until the end of the second quarter of 2009, an inability to raise capital could force us to go out of business. Declines in the price of our common stock or a failure of our stock price to increase sufficiently could also impair our ability to attract and retain qualified employees, reduce the liquidity of our common stock and result in the delisting of our common stock from the NASDAQ Capital Market. Even if our stock price increases sufficiently, we nonetheless expect to be delisted because of non-compliance with the $2.5 million shareholders equity requirement of Marketplace Rule 4310(c)(3) resulting from our goodwill impairment. If we are delisted from the NASDAQ Capital Market as we currently expect, our ability to raise additional capital through the equity markets will be substantially harmed.

Concentration of ownership of our common stock may give certain shareholders significant influence over our business and may result in certain decisions that are contrary to your interests.

A small number of investors own a significant number of shares of our common stock. As of March 31, 2009, Special Situations held approximately 2.5 million shares of our common stock, Biogen Idec held approximately 2.2 million shares, Elan International services, Ltd., or Elan, held approximately 1.2 million shares, and Renaissance Technologies held approximately 1.1 million shares. Together these holdings represent approximately 34% of our common shares outstanding as of March 31, 2009. This concentration of stock ownership may allow these shareholders to exercise significant control over our strategic decisions and block, delay or substantially influence all matters requiring shareholder approval, such as:

- approval of significant corporate transactions, such as a change of control of Targeted Genetics;
- election of directors; or
- amendment of our charter documents.

The interests of these shareholders may conflict with your interests or the interests of other holders of our common stock with regard to such matters. Furthermore, this concentration of ownership of our common stock could allow these shareholders to delay, deter or prevent a third party from acquiring control of us at a premium over the then-current market price of our common stock, which could result in a decrease in our stock price and a reduction in the value of your investment.

Special Situations, Biogen Idec, Elan and Renaissance Technologies have all sold shares of our common stock in the past and may continue to do so. Sales of significant value of stock by these investors may introduce increased volatility to the market price of our common stock.

Market fluctuations or volatility could cause the market price of our common stock to decline and limit our ability to raise capital or cause impairment issues.

The stock market in general and the market for biotechnology-related companies in particular have experienced extreme price and volume fluctuations, often unrelated to the operating performance of the affected companies. The market price of the securities of biotechnology companies, particularly companies such as ours without earnings and product revenue, has been highly volatile and is likely to remain so in the future. Any report of clinical trial results that are below the expectations of financial analysts or investors could result in a decline in our stock price. We believe that in the past, similar levels of volatility have contributed to the decline in the market price of our common stock, and may do so again in the future. Trading volumes of our common stock can increase dramatically, resulting in a volatile market price for our common stock. The trading price of our common stock could decline significantly as a result of sales of a substantial number of shares of our common stock, or the perception that significant sales could occur. In addition, the sale of significant quantities of stock by Special Situations, Biogen

Idec, Elan, Renaissance Technologies or other holders of significant amounts of shares of our stock could adversely impact the price of our common stock.

Table of Contents

Item 2. ***Unregistered Sales of Securities and Use of Proceeds***
None

Item 3. ***Defaults Upon Senior Securities***
None.

Item 4. ***Submission of Matters to a Vote of Security Holders***
None

Item 5. ***Other Information***
None.

Item 6. ***Exhibits***
See the Index to Exhibits included in this quarterly report.

Table of Contents

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

TARGETED GENETICS CORPORATION

Date: May 7, 2009

By: /s/ B.G. SUSAN ROBINSON
B.G. Susan Robinson,
President and Chief Executive Officer
(Principal Executive Officer)

Date: May 7, 2009

By: /s/ DAVID J. POSTON
David J. Poston,
Vice President, Finance and Chief Financial Officer
(Principal Financial and Accounting Officer)

Table of Contents

INDEX TO EXHIBITS

Exhibit Number	Exhibit Description	Form	Date of First Filing	Exhibit Number	Filed Herewith
3.1	Restated Articles of Incorporation	8-K	1/30/08	3.1	
3.2	Amended and Restated Bylaws.	8-K	12/28/07	3.1	
4.1	Registration Rights Agreement among Targeted Genetics Corporation and certain investors dated as of January 8, 2007	8-K	1/8/07	10.2	
4.2	Registration Rights Agreement among Targeted Genetics Corporation and certain purchasers dated as of June 22, 2007	8-K	6/25/07	10.2	
10.1	Amended and Restated Senior Management Employment Agreement, dated as of March 27, 2009, between Targeted Genetics Corporation and B.G. Susan Robinson	10-K	3/31/09	10.2	
10.2	License Agreement dated February 25, 2009, between Targeted Genetics Corporation and Celladon Corporation*				X
10.3	Amended and Restated Manufacturing Agreement dated February 25, 2009, between Targeted Genetics Corporation and Celladon Corporation*				X
10.4	Amended and Restated License Agreement dated January 29, 2009 between Targeted Genetics Corporation and The Trustees of the University of Pennsylvania*				X
31.1	Certification of Chief Executive Officer pursuant to Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended				X
31.2	Certification of Chief Financial Officer pursuant to Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended				X
32.1	Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002				X
32.2	Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002				X

* Portions of these exhibits have been omitted based on a grant of or application for confidential treatment from the SEC. The omitted portions of these exhibits have been filed separately with the SEC.