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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, DC 20549**

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**FORM 10-Q**

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(Mark One)

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

For the quarterly period ended September 30, 2013

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission File Number: 001-35966

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**bluebird bio, Inc.**

(Exact Name of Registrant as Specified in Its Charter)

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**Delaware**  
(State or Other Jurisdiction of  
Incorporation or Organization)

**13-3680878**  
(IRS Employer  
Identification No.)

**840 Memorial Drive, 4<sup>th</sup> Floor**  
**Cambridge, Massachusetts**  
(Address of Principal Executive Offices)

**02139**  
(Zip Code)

**(617) 491-5601**  
(Registrant's Telephone Number, Including Area Code)

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

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

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

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

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

As of October 31, 2013, there were 23,780,211 shares of the registrant's Common Stock, par value \$0.01 per share, outstanding.

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## FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “target,” “will,” “would,” or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the initiation, timing, progress and results of our preclinical and clinical studies, and our research and development programs;
- our ability to advance product candidates into, and successfully complete, clinical studies;
- our ability to advance our viral vector manufacturing and transduction capabilities;
- the timing or likelihood of regulatory filings and approvals;
- the commercialization of our product candidates, if approved;
- the pricing and reimbursement of our product candidates, if approved;
- the implementation of our business model, strategic plans for our business, product candidates and technology;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- estimates of our expenses, future revenues, capital requirements and our needs for additional financing;
- the potential benefits of strategic collaboration agreements and our ability to enter into strategic arrangements;
- our ability to maintain and establish collaborations or obtain additional grant funding;
- our financial performance;
- developments relating to our competitors and our industry; and
- other risks and uncertainties, including those listed under Part II, Item 1A. Risk Factors.

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Part II, Item 1A. Risk Factors and elsewhere in this Quarterly Report on Form 10-Q. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

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**bluebird bio, Inc.**  
**Form 10-Q**  
**For the Three and Nine Months Ended September 30, 2013**

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## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements

**bluebird bio, Inc.**  
**Condensed Consolidated Balance Sheets**  
**(unaudited)**  
**(in thousands, except per share data)**

|   | September 30,<br>2013 | December 31,<br>2012 |
|---|-----------------------|----------------------|
| <b>Assets</b>   |                       |                      |
| Current assets:   |                       |                      |
| Cash and cash equivalents   | \$ 216,791            | \$ 67,011            |
| Prepaid expenses and other current assets   | 4,676                 | 773                  |
| Total current assets  | 221,467               | 67,784               |
| Property and equipment, net   | 5,392                 | 1,288                |
| Restricted cash   | 1,403                 | 250                  |
| Total assets  | <u>\$ 228,262</u>     | <u>\$ 69,322</u>     |
| <b>Liabilities, convertible preferred stock, and stockholders' equity (deficit)</b>   |                       |                      |
| Current liabilities:  |                       |                      |
| Accounts payable  | \$ 1,144              | \$ 2,173             |
| Accrued expenses and other current liabilities  | 5,006                 | 2,115                |
| Deferred revenue, current portion   | 25,340                | 340                  |
| Total current liabilities   | 31,490                | 4,628                |
| Warrant liability   | —                     | 215                  |
| Deferred rent, net of current portion   | 2,529                 | 46                   |
| Deferred revenue, net of current portion  | 36,543                | 340                  |
| Total liabilities   | 70,562                | 5,229                |
| Commitments and contingencies ( <i>Note 5</i> )   |                       |                      |
| Series A-2 convertible preferred stock, \$0.01 par value, 0 and 22,304 shares authorized; 0 and 22,304 issued and outstanding at September 30, 2013 and December 31, 2012, respectively | —                     | 7,137                |
| Series B convertible preferred stock, \$0.01 par value, 0 and 115,779 shares authorized; 0 and 115,204 issued and outstanding at September 30, 2013 and December 31, 2012, respectively | —                     | 40,321               |
| Series C convertible preferred stock, \$0.01 par value, 0 and 39,943 shares authorized; 0 and 39,943 issued and outstanding at September 30, 2013 and December 31, 2012, respectively   | —                     | 12,382               |
| Series D convertible preferred stock, \$0.01 par value, 0 and 120,409 shares authorized; 0 and 120,409 issued and outstanding at September 30, 2013 and December 31, 2012, respectively | —                     | 60,000               |
| Stockholders' equity (deficit):   |                       |                      |
| Preferred stock \$0.01 par value, 5,000 and 0 shares authorized; 0 shares outstanding at September 30, 2013 and December 31, 2012, respectively   | —                     | —                    |
| Series A-1 convertible preferred stock, \$0.01 par value, 0 and 18,817 shares authorized; 0 and 12,981 issued and outstanding at September 30, 2013 and December 31, 2012, respectively | —                     | 2,337                |
| Common stock, \$0.01 par value, 125,000 and 21,511 shares authorized; 23,633 and 309 shares issued and outstanding at September 30, 2013 and December 31, 2012, respectively            | 236                   | 3                    |
| Additional paid-in capital  | 248,058               | 15,267               |
| Accumulated deficit   | (90,594)              | (73,354)             |
| Total stockholders' equity (deficit)  | 157,700               | (55,747)             |
| Total liabilities, convertible preferred stock and stockholders' equity (deficit)   | <u>\$ 228,262</u>     | <u>\$ 69,322</u>     |

*See accompanying notes to unaudited condensed consolidated financial statements.*

**bluebird bio, Inc.**  
**Condensed Consolidated Statements of Operations and Comprehensive Loss**  
**(unaudited)**  
**(in thousands, except per share data)**

|   | Three months ended<br>September 30, |                   | Nine months ended<br>September 30, |                    |
|---|-------------------------------------|-------------------|------------------------------------|--------------------|
|   | 2013                                | 2012              | 2013                               | 2012               |
| Revenue:  |                                     |                   |                                    |                    |
| Collaboration revenue   | \$ 6,251                            | \$ —              | \$ 13,542                          | \$ —               |
| Research and license fees   | 134                                 | 85                | 304                                | 255                |
| Total revenue   | <u>6,385</u>                        | <u>85</u>         | <u>13,846</u>                      | <u>255</u>         |
| Operating expenses:   |                                     |                   |                                    |                    |
| Research and development  | 8,706                               | 3,501             | 21,237                             | 10,713             |
| General and administrative  | 3,836                               | 1,608             | 9,441                              | 4,302              |
| Total operating expenses  | <u>12,542</u>                       | <u>5,109</u>      | <u>30,678</u>                      | <u>15,015</u>      |
| Loss from operations  | (6,157)                             | (5,024)           | (16,832)                           | (14,760)           |
| Other income (expense), net:  |                                     |                   |                                    |                    |
| Interest income   | 11                                  | 1                 | 21                                 | 3                  |
| Foreign currency gains  | 33                                  | 4                 | 11                                 | 18                 |
| Re-measurement of warrants  | —                                   | (22)              | (440)                              | 67                 |
| Other income (expense), net   | 44                                  | (17)              | (408)                              | 88                 |
| Net loss  | <u>\$ (6,113)</u>                   | <u>\$ (5,041)</u> | <u>\$ (17,240)</u>                 | <u>\$ (14,672)</u> |
| Net (loss) income applicable to common stockholders—basic   | <u>\$ (6,113)</u>                   | <u>\$ 315</u>     | <u>\$ (17,240)</u>                 | <u>\$ 111</u>      |
| Net (loss) income applicable to common stockholders—diluted   | <u>\$ (6,113)</u>                   | <u>\$ 632</u>     | <u>\$ (17,240)</u>                 | <u>\$ 243</u>      |
| Net (loss) income per share applicable to common stockholders:  |                                     |                   |                                    |                    |
| Basic   | <u>\$ (0.26)</u>                    | <u>\$ 1.15</u>    | <u>\$ (1.96)</u>                   | <u>\$ 0.44</u>     |
| Diluted   | <u>\$ (0.26)</u>                    | <u>\$ 1.14</u>    | <u>\$ (1.96)</u>                   | <u>\$ 0.44</u>     |
| Weighted-average number of common shares used in computing net (loss) income per share applicable to common stockholders: |                                     |                   |                                    |                    |
| Basic   | <u>23,623</u>                       | <u>275</u>        | <u>8,786</u>                       | <u>250</u>         |
| Diluted   | <u>23,623</u>                       | <u>554</u>        | <u>8,786</u>                       | <u>551</u>         |
| Comprehensive loss  | <u>\$ (6,113)</u>                   | <u>\$ (5,041)</u> | <u>\$ (17,240)</u>                 | <u>\$ (14,672)</u> |

*See accompanying notes to unaudited condensed consolidated financial statements.*

**bluebird bio, Inc.**  
**Condensed Consolidated Statements of Cash Flows**  
**(unaudited)**  
**(in thousands)**

|   | <u>Nine months ended September 30,</u> |                  |
|---|--|------------------|
|   | <u>2013</u>                            | <u>2012</u>      |
| <b>Operating activities</b>   |  |                  |
| Net loss  | \$ (17,240)                            | \$ (14,672)      |
| Adjustments to reconcile net loss to net cash used in operating activities:           |  |                  |
| Depreciation and amortization   | 610                                    | 205              |
| Stock-based compensation expense  | 4,833                                  | 570              |
| Re-measurement of warrants  | 440                                    | (67)             |
| Loss on disposal of equipment   | 2                                      | —                |
| Changes in operating assets and liabilities:  |  |                  |
| Prepaid expenses and other current assets   | (3,903)                                | (218)            |
| Accounts payable  | (1,021)                                | (755)            |
| Accrued expenses and other liabilities  | 3,981                                  | 784              |
| Deferred revenue  | 61,203                                 | (255)            |
| Net cash provided by (used in) operating activities                                   | <u>48,905</u>                          | <u>(14,408)</u>  |
| <b>Investing activities</b>   |  |                  |
| Restricted cash   | (1,153)                                | (40)             |
| Purchase of property and equipment  | (3,380)                                | (511)            |
| Proceeds from sales or maturities of marketable securities                            | —                                      | 3,506            |
| Net cash (used in) provided by investing activities                                   | <u>(4,533)</u>                         | <u>2,955</u>     |
| <b>Financing activities</b>   |  |                  |
| Proceeds from IPO, net of issuance costs  | 104,972                                | —                |
| Proceeds from issuance of Series D preferred stock, net                               | —                                      | 59,859           |
| Repayment of nonrecourse note collateralized by restricted stock                      | 344                                    | —                |
| Proceeds from exercise of stock options   | 92                                     | 8                |
| Net cash provided by financing activities   | <u>105,408</u>                         | <u>59,867</u>    |
| Increase in cash and cash equivalents   | 149,780                                | 48,414           |
| Cash and cash equivalents at beginning of period                                      | 67,011                                 | 25,604           |
| Cash and cash equivalents at end of period  | <u>\$ 216,791</u>                      | <u>\$ 74,018</u> |
| <b>Non-cash investing and financing activities:</b>                                   |  |                  |
| Purchases of property and equipment included in accounts payable and accrued expenses | \$ 1,347                               | \$ 2             |
| Accretion and dividends on convertible preferred stock                                | \$ —                                   | \$ 3,057         |
| Gain on extinguishment of convertible preferred stock                                 | \$ —                                   | \$ 23,114        |
| Reclassification of warrants to additional paid-in capital                            | \$ 655                                 | \$ 394           |
| Reclassification of Series A-1 Preferred Stock to common stock                        | \$ —                                   | \$ 2,337         |
| Conversion of preferred stock to common stock upon closing of IPO                     | \$ 122,177                             | \$ —             |

*See accompanying notes to unaudited condensed consolidated financial statements.*

**bluebird bio, Inc.**

**Notes to Condensed Consolidated Financial Statements  
(In thousands, except per share data)  
(unaudited)**

**1. Description of the business**

bluebird bio, Inc. (the “Company”) was incorporated in Delaware on April 16, 1992, and is headquartered in Cambridge, Massachusetts. The Company was formed to develop, manufacture and market therapies to safely and effectively deliver genes useful in the treatment of serious human diseases. Since its inception, the Company has devoted substantially all of its resources to its development efforts relating to its product candidates, including activities to manufacture product in compliance with good manufacturing practices (“GMP”), preparing to conduct clinical studies of its product candidates, providing general and administrative support for these operations and protecting its intellectual property.

**2. Summary of significant accounting policies and basis of presentation**

***Initial public offering***

On June 24, 2013, the Company completed its initial public offering (“IPO”) whereby the Company sold 6,832 shares of common stock (inclusive of 891 shares of common stock sold by the Company pursuant to the full exercise of an overallotment option granted to the underwriters in connection with the offering) at a price of \$17.00 per share. The shares began trading on the Nasdaq Global Select Market on June 19, 2013. The aggregate net proceeds received by the Company from the offering were \$104,921, net of underwriting discounts and commissions and estimated offering expenses payable by the Company. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 16,389 shares of common stock; and warrants exercisable for convertible preferred stock were automatically converted into warrants exercisable for 338 shares of common stock, resulting in the reclassification of the related convertible preferred stock warrant liability of \$655 to additional paid-in capital. Additionally, the Company is now authorized to issue 125,000 shares of common stock and 5,000 shares of preferred stock.

***Basis of presentation and principles of consolidation***

The accompanying condensed consolidated financial statements are unaudited and have been prepared by the Company in accordance with accounting principles generally accepted in the United States (“GAAP”) as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Update (“ASU”) of the Financial Accounting Standards Board (“FASB”). Certain information and footnote disclosures normally included in the Company’s annual financial statements have been condensed or omitted. These interim condensed consolidated financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the Company’s financial position and results of operations for the interim periods ended September 30, 2013 and 2012.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These interim financial statements should be read in conjunction with the audited financial statements as of and for the year ended December 31, 2012, and the notes thereto, which are included in the Company’s Prospectus that forms a part of the Company’s Registration Statement on Form S-1 (File No. 333-188605), which was filed with the Securities and Exchange Commission (the “SEC”) pursuant to Rule 424 on June 19, 2013 (the “Prospectus”).

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, bluebird bio France, SARL and bluebird bio Securities Corporation. All intercompany balances and transactions have been eliminated in consolidation. Any reference in these notes to applicable guidance is meant to refer to GAAP. The Company views its operations and manages its business in one operating segment. All material long-lived assets of the Company reside in the United States.

***Reverse stock split***

On June 3, 2013, the board of directors and the stockholders of the Company approved a one-for-18.967 reverse stock split of the Company’s outstanding common stock, which was effected on June 3, 2013. Stockholders entitled to fractional shares as a result of the reverse stock split will receive a cash payment in lieu of receiving fractional shares. The Company’s historical share and per share information have been retroactively adjusted to give effect to this reverse stock split. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices, if applicable, were proportionately increased in accordance with the terms of the agreements governing such securities.

### **Summary of accounting policies**

The significant accounting policies described in the Company's audited financial statements as of and for the year ended December 31, 2012, and the notes thereto, which are included in the Prospectus, have had no material changes during the nine months ended September 30, 2013, except as noted below:

#### *Collaboration revenue*

As of September 30, 2013, the Company's collaboration revenue is generated exclusively from its collaboration arrangement with Celgene Corporation ("Celgene"). The terms of this arrangement contain multiple deliverables, which include at inception: (i) discovery, research and development services, (ii) participation on the joint steering committee and (iii) participation on the patent committee. The collaboration arrangement also provides Celgene with the option to obtain a license to any product candidates resulting from the collaboration. Moreover, Celgene has the option to extend the term of the collaboration arrangement, first for a period of two years and then for an additional period of one year. Additionally, the Company has the sole right to manufacture or have manufactured supplies of vectors and associated payloads manufactured for incorporation into the associated product candidate in the event a product candidate is licensed. Non-refundable payments to the Company under this arrangement may include: (i) up-front research fees, (ii) product candidate license fees, (iii) extension term research fees, (iv) payments for the manufacture and supply of vectors and payloads, (v) payments based on the achievement of certain milestones and (vi) royalties on product sales. Additionally, the Company may elect to share in the costs incurred from the development, commercialization and manufacture of product candidates licensed by its collaborators and earn its share of the net profits or bear its share of the net losses generated from the sale of product candidates licensed by its collaborators.

The Company analyzes multiple-element arrangements based on the guidance in FASB ASC Topic 605-25, *Revenue Recognition-Multiple-Element Arrangements* ("ASC 605-25"). Pursuant to the guidance in ASC 605-25, the Company evaluates multiple-element arrangements to determine (1) the deliverables included in the arrangement and (2) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially in the control of the Company. In assessing whether an item has standalone value, the Company considers factors such as the research, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can use the other deliverable(s) for their intended purpose without the receipt of the remaining element(s), whether the value of the deliverable is dependent on the undelivered item(s) and whether there are other vendors that can provide the undelivered element(s). The Company's collaboration arrangement does not contain a general right of return relative to the delivered item(s).

Arrangement consideration that is fixed or determinable is allocated among the separate units of accounting using the relative selling price method. Then, the applicable revenue recognition criteria in ASC 605-25 are applied to each of the separate units of accounting in determining the appropriate period and pattern of recognition. The Company determines the selling price of a unit of accounting following the hierarchy of evidence prescribed by ASC 605-25. Accordingly, the Company determines the estimated selling price for units of accounting within each arrangement using vendor-specific objective evidence ("VSOE") of selling price, if available, third-party evidence ("TPE") of selling price if VSOE is not available, or best estimate of selling price ("BESP") if neither VSOE nor TPE is available. The Company typically uses BESP to estimate the selling price, since it generally does not have VSOE or TPE of selling price for its units of accounting. Determining the BESP for a unit of accounting requires significant judgment. In developing the BESP for a unit of accounting, the Company considers applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates the BESP for units of accounting by evaluating whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

Options are considered substantive if, at the inception of the arrangement, the Company is at risk as to whether the collaboration partner will choose to exercise the option. Factors that the Company considers in evaluating whether an option is substantive include the overall objective of the arrangement, the benefit the collaborator might obtain from the arrangement without exercising the option, the cost to exercise the option and the likelihood that the option will be exercised. For arrangements under which an option is considered substantive, the Company does not consider the item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable arrangement consideration, assuming the option is not priced at a significant and incremental discount. Conversely, for arrangements under which an option is not considered substantive or if an option is priced at a significant and incremental discount, the Company would consider the item underlying the option to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. All of the options included in the Company's collaboration arrangement have been determined to be substantive, and none of the options are priced at a significant and incremental discount.



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The Company recognizes arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in ASC 605-25 are satisfied for that particular unit of accounting. The Company will recognize as revenue arrangement consideration attributed to licenses that have standalone value from the other deliverables to be provided in an arrangement upon delivery. The Company will recognize as revenue arrangement consideration attributed to licenses that do not have standalone value from the other deliverables to be provided in an arrangement over the Company's estimated performance period as the arrangement would be accounted for as a single unit of accounting.

The Company recognizes revenue from the Celgene arrangement associated with discovery, research and development services, joint steering committee services and patent committee services ratably over the associated period of performance. If there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, then the Company recognizes revenue under the arrangement on a straight-line basis over the period the Company is expected to complete its performance obligations. Conversely, if the pattern of performance in which the service is provided to the customer can be determined and objectively measurable performance measures exist, then the Company recognizes revenue under the arrangement using the proportional performance method. Revenue recognized is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the straight-line method or proportional performance method, as applicable, as of the period ending date.

At the inception of an arrangement that includes milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (ii) the consideration relates solely to past performance and (iii) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. The Company evaluates factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestone and the level of effort and investment required to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. The Company has concluded that all of the clinical and regulatory milestones pursuant to its collaboration arrangement are substantive. Accordingly, in accordance with FASB ASC Topic 605-28, *Revenue Recognition-Milestone Method*, revenue from clinical and regulatory milestone payments will be recognized in its entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive would be recognized as revenue over the remaining period of performance, assuming all other revenue recognition criteria are met. Revenue from commercial milestone payments will be accounted for as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

The Company will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

### *Use of estimates*

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could materially differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the financial statements. Estimates are used in the following areas, among others: stock-based compensation expense, accrued expenses, valuation of warrants, revenue and income taxes.

### ***Fair value of financial instruments***

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. FASB ASC Topic 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.

Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable. To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Items measured at fair value on a recurring basis include the warrant liability (Note 4). The carrying amounts of accounts payable and accrued expenses approximate their fair values due to their short-term maturities.

### ***Net Income (Loss) Per Share***

The Company follows the two-class method when computing net income (loss) per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. Accordingly, in periods in which the Company reports a net loss attributable to common stockholders, losses are not allocated to participating securities.

Basic net income (loss) per share is calculated by dividing net income (loss) attributable to common stockholders by the weighted average number of common shares outstanding during the period. Diluted net income per share is calculated by dividing the net income attributable to common stockholders by the weighted-average number of common equivalent shares outstanding for the period.

Diluted net income per share includes any dilutive effect from outstanding stock options and warrants using the treasury stock method.

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### 3. Cash and cash equivalents

The Company considers all highly liquid securities with original final maturities of three months or less from the date of purchase to be cash equivalents. As of September 30, 2013 and December 31, 2012, cash and cash equivalents comprise funds in cash and money market accounts. The following table presents the cash and cash equivalents carried at fair value:

| <u>Description</u>              | <u>Total</u>     | <u>Quoted prices in<br/>active markets<br/>(Level 1)</u> | <u>Significant other<br/>observable inputs<br/>(Level 2)</u> | <u>Significant<br/>unobservable inputs<br/>(Level 3)</u> |
|---------------------------------|------------------|--|--|--|
| <b>September 30, 2013</b>       |                  |  |  |  |
| Cash held in banks              | \$ 3,774         | \$ 3,774   | \$ —   | \$ —   |
| Money market funds              | 213,017          | 213,017  | —  | —  |
| Total cash and cash equivalents | <u>\$216,791</u> | <u>\$ 216,791</u>  | <u>\$ —</u>  | <u>\$ —</u>  |
| <b>December 31, 2012</b>        |                  |  |  |  |
| Cash held in banks              | \$ 14,011        | \$ 14,011  | \$ —   | \$ —   |
| Money market funds              | 53,000           | 53,000   | —  | —  |
| Total cash and cash equivalents | <u>\$ 67,011</u> | <u>\$ 67,011</u>   | <u>\$ —</u>  | <u>\$ —</u>  |

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**4. Warrants**

As of December 31, 2012, the Company had outstanding warrants to purchase 6,512 shares of capital stock. Upon the closing of the IPO on June 24, 2013, all of the warrants exercisable for convertible preferred stock were automatically converted into warrants exercisable for 338 shares of common stock. The warrants outstanding consist of the following:

|   | <u>September 30, 2013</u> | <u>December 31, 2012</u> |
|---|---------------------------|--------------------------|
| Warrants to purchase Series A-1 Preferred Stock | —                         | 5,835                    |
| Warrants to purchase Series B Preferred Stock   | —                         | 575                      |
| Warrants to purchase Common Stock               | 440                       | 102                      |
|   | <u>440</u>                | <u>6,512</u>             |

In conjunction with the closing of the Company's IPO, all warrants exercisable for convertible preferred stock were automatically converted into warrants exercisable for common stock, resulting in the reclassification of the related convertible preferred stock warrant liability to additional paid-in capital as warrants to purchase shares of common stock are accounted for as equity instruments. The warrant liability was re-measured to fair value prior to reclassification to additional paid-in capital. As of September 30, 2013, the Company had no outstanding warrant liability. The warrant liability measured at fair value as of December 31, 2012 is as follow:

| <u>Description</u>       | <u>Total</u> | <u>Quoted prices in<br/>active markets<br/>(Level 1)</u> | <u>Significant other<br/>observable inputs<br/>(Level 2)</u> | <u>Significant<br/>unobservable inputs<br/>(Level 3)</u> |
|--------------------------|--------------|--|--|--|
| <b>December 31, 2012</b> |              |  |  |  |
| Warrant liability        | \$215        | \$ —   | \$ —   | \$ 215   |
|                          | <u>\$215</u> | <u>\$ —</u>  | <u>\$ —</u>  | <u>\$ 215</u>  |

The following table sets forth a summary of changes in the fair value of the Company's preferred stock warrant liability which represents a recurring measurement that is classified within Level 3 of the fair value hierarchy wherein fair value is estimated using significant unobservable inputs:

|                            | <u>Nine months ended<br/>September 30, 2013*</u> |
|----------------------------|--|
| Beginning balance          | \$ 215   |
| Change in fair value       | 440  |
| Reclassification to equity | (655)  |
| Ending balance             | <u>\$ —</u>                                      |

\* These warrants were re-measured to fair value and then reclassified to additional paid-in capital on June 24, 2013.

The fair value of each warrant to purchase shares of the Company's Series A-1 Preferred Stock as of September 30, 2012 was estimated using the Black-Scholes option pricing model with the following weighted-average assumptions:

|                                     | <u>Three months ended<br/>September 30, 2012**</u> | <u>Nine months ended<br/>September 30, 2012**</u> |
|-------------------------------------|--|---|
| Fair value of underlying instrument | \$ 0.18  | \$ 0.18   |
| Expected volatility                 | 78.9%  | 78.9%   |
| Expected term (in years)            | 4.98   | 4.98  |
| Risk-free interest rate             | 0.6%   | 0.6%  |
| Expected dividend yield             | 0.0%   | 0.0%  |

\*\* Series A-1 warrants were re-measured to fair value and then reclassified to additional paid-in capital on July 23, 2012.

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The fair value of each warrant to purchase shares of the Company's Series B Preferred Stock was estimated using the Black-Scholes option pricing model with the following weighted-average assumptions:

|                                     | Three months ended | Nine months ended |         |
|-------------------------------------|--------------------|-------------------|---------|
|                                     | September 30,      | September 30,     |         |
|                                     | 2012               | 2013*             | 2012    |
| Fair value of underlying instrument | \$ 0.41            | \$0.95            | \$ 0.45 |
| Expected volatility                 | 79.7%              | 82.0%             | 77.6%   |
| Expected term (in years)            | 6.54               | 5.93              | 6.79    |
| Risk-free interest rate             | 0.9%               | 1.1%              | 1.2%    |
| Expected dividend yield             | 0.0%               | 0.0%              | 0.0%    |

\* Series B warrants were re-measured to fair value and then reclassified to additional paid-in capital on June 24, 2013.

## 5. Commitments and contingencies

On June 3, 2013, the Company entered into a new nine-year building lease for approximately 43,600 square feet of space in Cambridge, Massachusetts, commencing on the earlier of the substantial completion of the build-out work or January 1, 2014. The lease has monthly lease payments of \$209 the first 12 months with annual rent escalations thereafter and provides a rent abatement of \$209 per month for the first six months. The Company has the option to extend this lease by an additional five years. As the Company obtained access to the newly leased space on July 22, 2013 in order to begin the build-out, this is considered the lease commencement date for accounting purposes, thus rent expense began on this date and will be recognized on a straight-line basis over the term of the lease. In addition, the lease provides a contribution from the landlord towards the initial build-out of the space of up to \$6,538. The Company capitalizes the leasehold improvements as property and equipment and records the landlord incentive payments received as deferred rent and amortizes these amounts as reductions to rent expense over the lease term. The total operating lease obligation of the non-cancelable lease term of this agreement is \$24,209. Future minimum annual lease payments as of September 30, 2013, under this non-cancelable operating lease through the end of the lease term are as follow: \$1,253 in 2014; \$2,582 in 2015; \$2,659 in 2016; \$2,739 in 2017; \$2,821 in 2018; and \$12,155 in the aggregate thereafter. In accordance with the lease, the Company entered into a cash-collateralized irrevocable standby letter of credit in the amount of \$1,253, naming the landlord as beneficiary. This letter of credit is reduced to \$1,044, \$835, and \$627 upon the rent commencement date and the first and second anniversaries of the rent commencement date, respectively. The Company's current building lease in Cambridge, Massachusetts, expires on March 31, 2015. The Company plans to relocate to its new facility prior to the expiration of the lease for its current facility. The Company plans to sublease its current facility for the remainder of the lease term; however, it may be unable to sublease this facility or may enter into a sublease that does not provide the Company with funds sufficient to cover the Company's lease obligations. In the event that a sublease is signed, the rent abatement on the new lease may decrease.

The Company is party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at September 30, 2013 and December 31, 2012 or royalties on future sales of specified products.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company indemnifies, holds harmless, and agrees to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners or customers, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third party with respect to the Company's products. The term of these indemnification agreements is generally perpetual any time after execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

## 6. Convertible preferred stock

Upon the closing of the IPO on June 24, 2013, all of the outstanding shares of the Company's convertible preferred stock were converted into 16,389 shares of its common stock. As of September 30, 2013, the Company does not have any convertible preferred stock issued or outstanding.

## 7. Significant agreements

### *Celgene Corporation*

#### *Summary of the Collaboration Agreement*

On March 19, 2013, the Company entered into a Master Collaboration Agreement (the "Collaboration Agreement") with Celgene to discover, develop and commercialize disease-altering gene therapies in oncology. The collaboration is focused on applying gene therapy technology to genetically modify a patient's own T cells, known as chimeric antigen receptor, or CAR, T cells, to target and destroy cancer cells. Additionally, on March 19, 2013, the Company entered into a Platform Technology Sublicense Agreement (the "Sublicense Agreement") with Celgene pursuant to which the Company obtained a sublicense to certain intellectual property from Celgene, originating under Celgene's license from Baylor College of Medicine, for use in the collaboration.

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Under the terms of the Collaboration Agreement, the Company received a \$75,000 up-front, non-refundable cash payment. The Company will be responsible for conducting discovery, research and development activities through completion of Phase I clinical studies, if any, during the initial term of the agreement, or three years. The collaboration will be governed by a joint steering committee (“JSC”) formed by an equal number of representatives from the Company and Celgene. The JSC will, among other activities, review the collaboration program, review and evaluate product candidates and approve regulatory plans. In addition to the JSC, the Collaboration Agreement provides that the Company and Celgene will each appoint representatives to establish a patent committee, which will be responsible for managing the intellectual property developed and used during the collaboration.

Prior to expiration of the initial term of the Collaboration Agreement, Celgene has two options to extend the term, through March 19, 2019, with the payment of significant extension fees. Separately, Celgene has an option to license an unlimited number of product candidates resulting from the collaboration during a period commencing upon execution of the Collaboration Agreement and continuing through a specified period following the completion of Phase I clinical studies for each individual product candidate. In the event such option is exercised, the Company would grant Celgene an exclusive worldwide license to develop and commercialize such product candidate. Upon exercise of the option to license a product candidate, Celgene is required to pay an option fee, which is subject to reduction if the Company elects to co-develop and co-promote such product candidate in the United States. For any product candidates licensed by Celgene, the Company may be responsible, at Celgene’s election, to continue performing certain development activities contemplated as part of the collaboration plan. If Celgene does not exercise its option with respect to a product candidate prior to the expiration of the applicable option period (each a “declined product candidate”), then the Company has the right to develop the product candidate outside the scope of the collaboration, subject to a Celgene opt-in right to obtain a license to that declined product candidate for significant additional cash consideration. The opt-in right exists through a specified period following the completion of a pivotal study for the specific declined product candidate and functions in the same manner as the option to license any other product candidates resulting from the collaboration.

In addition, Celgene would be required to make certain milestone payments upon the achievement of specified clinical, regulatory and commercial events. For each product candidate that is licensed by Celgene, the Company would be eligible to receive per product up to \$20,000 in option fees, up to \$10,000 in clinical milestone payments, up to \$117,000 in regulatory milestone payments and up to \$78,000 in commercial milestone payments. Clinical milestone payments are triggered upon initiation of a defined phase of clinical research for a product candidate. Regulatory milestone payments are triggered upon approval to market a product candidate by the FDA or other global regulatory authorities. Commercial milestone payments are triggered upon the first commercial sale of an approved pharmaceutical product and when an approved pharmaceutical product reaches certain defined levels of net sales by the licensee or receives approval to be marketed by certain global regulatory authorities in a specified number of countries outside of the United States. In addition, to the extent any of the product candidates licensed by Celgene are commercialized, the Company would be entitled to receive tiered royalty payments ranging from the mid-single digits to mid-teens based on a percentage of net sales. Royalty payments are subject to certain reductions, including for any royalty payments required to be made by Celgene to acquire patent rights, with an aggregate minimum floor. The Company is not eligible to receive either milestone payments or royalty payments unless and until Celgene exercises its option to license a product candidate resulting from the collaboration whereupon the parties will execute a license agreement, the terms of which are included as part of the collaboration arrangement.

Additionally, the Company may elect to co-develop and co-promote product candidates licensed by Celgene. If the Company elects to co-develop and co-promote a product candidate, then the parties would share equally in all costs incurred relating to the development, commercialization and manufacture of the product candidate within the United States and share equally in the profits generated by such product candidate in the United States. Additionally, if the Company elects to co-develop and co-promote a product candidate, then the option fees, milestones and royalties would decrease compared to those described above. Under this scenario, the Company would receive per product up to \$10,000 in option fees, up to \$10,000 in clinical milestone payments and outside of the United States, up to \$54,000 in regulatory milestone payments and up to \$36,000 in commercial milestone payments. Clinical milestone payments are triggered upon initiation of a defined phase of clinical research for a product candidate. Regulatory milestone payments are triggered upon approval to market a product candidate by global regulatory authorities. Commercial milestone payments are triggered when an approved pharmaceutical product reaches certain defined levels of net sales by the licensee or receives approval to be marketed by certain global regulatory authorities in a specified number of countries outside the United States. In addition, to the extent any of the product candidates licensed by Celgene and co-developed and co-promoted by the Company are commercialized, the Company would be entitled to receive tiered royalty payments ranging from the mid-single digits to mid-teens based on a percentage of net sales from sales generated outside of the United States. Royalty payments are subject to certain reductions, including for any royalty payments required to be made by Celgene to acquire patent rights, with an aggregate minimum floor. The Company is not eligible to receive profit share payments, milestone payments or royalty payments unless and until Celgene exercises its option to license a product candidate resulting from the collaboration whereupon the parties will execute a co-development, co-promote and profit share agreement, the terms of which are included as part of the collaboration arrangement.

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In the event Celgene elects to license a product candidate discovered and developed as part of the Collaboration Agreement, Celgene would be solely responsible for all costs and expenses of manufacturing and supplying any product candidates. Subject to customary back-up supply rights granted to Celgene, the Company has the sole right to manufacture or have manufactured supplies of vectors and associated payloads manufactured for incorporation into the associated product candidate. Celgene would reimburse the Company for the costs incurred to manufacture and supply such vectors and associated payloads, plus a modest mark-up. The Company is not obligated to manufacture or have manufactured supplies of vectors and associated payloads for incorporation into an optioned product candidate unless and until Celgene exercises its option to license a product candidate resulting from the collaboration whereupon the parties will execute a separate manufacturing and supply agreement.

The Collaboration Agreement may be terminated by either the Company or Celgene, upon written notice, in the event of the other party's uncured material breach. Celgene may terminate the Collaboration Agreement for any reason upon written notice to the Company. If the Collaboration Agreement is terminated, rights to product candidates in development at the time of such termination will be allocated to the parties through a mechanism included in the Collaboration Agreement. In addition, if Celgene terminates the Collaboration Agreement as a result of a breach by the Company, then any then-existing co-development and co-promotion agreement will be automatically terminated and replaced with a license agreement for such product candidate and any amounts payable by Celgene under any then-existing product license agreements will be reduced.

### *Call Option*

During the initial three-year term of the collaboration and, if extended, during the first two-year extension term of the collaboration, in the event that the Company engages in a change in control transaction, including for such purposes a merger or consolidation of the Company or the sale of all or substantially all of the Company's assets, or if another person or entity or group of persons or entities acquires at least 50% of the Company's voting capital stock, then Celgene has the right, but not the obligation, to terminate the Collaboration Agreement and obtain perpetual, non-terminable, worldwide, exclusive, fully paid-up licenses to all, but not less than all, of the product candidates previously identified under the Collaboration Agreement (the "Call Option"). Under the Call Option, the product candidates to which Celgene would have the right to acquire licenses include any product candidate previously licensed out of the collaboration during the term of the collaboration, any product candidate for which the Company has exercised the right to co-develop and co-promote within the United States, any product candidate for which Celgene previously declined its option to obtain a license and any product candidate for which at least *in vivo* efficacy studies have been initiated or authorized by the JSC. The purchase price for such licenses would be based on the fair value of these rights received and obligations assumed determined pursuant to a binding arbitration process.

In addition, during the initial three-year term of the collaboration, but not during any extension term, in the event that Celgene exercises the Call Option, in addition to the right to acquire the fully paid-up licenses described above, Celgene would obtain a perpetual, non-terminable, worldwide, exclusive license to the Company's intellectual property to develop one or more CAR T cell products targeting one or more oncology associated target antigens for the remainder of the initial collaboration term. Following the initial collaboration term, the license to the Company's intellectual property is limited to target antigens identified by Celgene promptly following the initial collaboration term for which Celgene reasonably intends to develop CAR T cell products. There is no limit to the number of oncology-related target antigens Celgene may select under this license. Upon commercialization of any such product candidate so licensed by Celgene, Celgene would be obligated to pay the Company a specified milestone payment upon regulatory approval and a percentage of net sales as a royalty.

The Company has concluded that the value of the Call Option is immaterial based primarily on the probability that the Call Option would become exercisable.

### *Accounting Analysis*

The Company's arrangement with Celgene contains the following deliverables: (i) discovery, research and development services, (ii) participation on the JSC and (iii) participation on the patent committee. The Company has determined that the options to extend the term of the agreement and the options to license product candidates, including those related to Celgene's opt-in right for a declined product candidate, are substantive options. Celgene is not contractually obligated to exercise the options. Additionally, as a result of the uncertain outcome of the discovery, research and development activities, the Company is at risk with regard to whether Celgene will exercise the options. Moreover, the Company has determined that the options are not priced at a significant and incremental discount. Accordingly, the options are not considered deliverables at the inception of the arrangement and the associated option fees are not included in allocable arrangement consideration. The Company has determined that the potential obligation to manufacture or have manufactured supplies of vectors and associated payloads for incorporation into an optioned product candidate is contingent upon Celgene exercising its option to license a product candidate resulting from the collaboration. Therefore, consistent with the treatment of the options to license product candidates, the Company's potential obligation under a manufacturing and supply agreement is not considered a deliverable at the inception of the arrangement and the associated fees are not included in allocable arrangement consideration.

The Company has concluded that each of the three deliverables identified at the inception of the arrangement (discovery, research and development services, participation on the JSC and participation on the patent committee) has standalone value from the other undelivered elements. Additionally, the Collaboration Agreement does not include return rights related to the initial collaboration term. Accordingly, each deliverable qualifies as a separate unit of accounting.



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The Company has identified the allocable arrangement consideration as the \$75,000 up-front payment. The Company determined that each of the identified deliverables have the same period of performance (the three year initial term) and have the same pattern of revenue recognition, ratably over the period of performance. As a result, the \$75,000 arrangement consideration will be recognized over the three year initial term.

The Company has evaluated all of the milestones that may be received in connection with Celgene's option to license a product candidate resulting from the collaboration. In evaluating if a milestone is substantive, the Company assesses whether: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (ii) the consideration relates solely to past performance and (iii) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. All clinical and regulatory milestones are considered substantive on the basis of the contingent nature of the milestone, specifically reviewing factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the milestone as well as the level of effort and investment required. Accordingly, such amounts will be recognized as revenue in full in the period in which the associated milestone is achieved, assuming all other revenue recognition criteria are met. All commercial milestones will be accounted for in the same manner as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

During the three and nine months ended September 30, 2013, the Company recognized \$6,251 and \$13,542, respectively, of revenue associated with its collaboration with Celgene related to the recognition of discovery, research and development services. As of September 30, 2013, there is \$61,458 of deferred revenue related to the Company's collaboration with Celgene which is classified as current or long-term in the accompanying balance sheet based on the contractual term of the arrangement.

### *Association Française contre les Myopathies*

In January 2011, the Company entered into a research funding agreement with the Association Française contre les Myopathies ("AFM"), a nonprofit organization dedicated to curing rare neuromuscular diseases and providing treatments to reduce the associated disabilities of such diseases. As part of the agreement, AFM funded the Company 1,000 Euros to be used to advance the Company's research, process development, manufacturing, preclinical development, and clinical development in gene therapy for beta-hemoglobinopathies in  $\beta$ -thalassemia and/or in Sickle Cell Disease.

The funding, or a portion thereof depending on timing, shall be repaid to AFM upon any of the following events: (i) upon out-licensing or sale of the program, (ii) upon obtaining the first product authorization for the market, or (iii) upon sale of the Company, provided that the development is active at the time of such sale. The agreement is for a period of four years. The Company believes that repayment of the funds paid under the agreement is not probable at the date of the agreement, September 30, 2013 or December 31, 2012. The Company recognizes the revenue under this arrangement on a straight-line basis over the term of the agreement. The Company will reassess the probability of repayment at the end of each reporting period.

### **8. Stock-based compensation**

On June 3, 2013, the Company's board of directors adopted its 2013 Stock Option and Incentive Plan ("2013 Plan"), which was subsequently approved by its stockholders and became effective upon the closing of the Company's initial public offering on June 24, 2013. The 2013 Plan replaces the 2010 Stock Option and Grant Plan ("2010 Plan").

The 2013 Plan allows for the granting of incentive stock options, non-qualified stock options, and restricted stock awards to the Company's employees, members of the board of directors, and consultants of the Company. The Company initially reserved 955 shares of its common stock for the issuance of awards under the 2013 Plan. The 2013 Plan provides that the number of shares reserved and available for issuance under the 2013 Plan will automatically increase each January 1, beginning on January 1, 2014, by four percent of the outstanding number of shares of common stock on the immediately preceding December 31 or such lesser number of shares as determined by the Company's compensation committee.

Any options or awards outstanding under the Company's previous stock option plans, including both the 2010 Plan and the Second Amended and Restated 2002 Employee, Director and Consultant Stock Plan, at the time of adoption of the 2013 Plan remain outstanding and effective. As of September 30, 2013, the total number of common shares that may be issued under all equity award plans is 5,282 and approximately 858 remain available for future grants.



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**Stock-based compensation expense**

Stock-based compensation by award type is as follows:

|                         | Three months ended September 30, |               | Nine months ended September 30, |               |
|-------------------------|----------------------------------|---------------|---------------------------------|---------------|
|                         | 2013                             | 2012          | 2013                            | 2012          |
| Stock options           | \$ 2,495                         | \$ 167        | \$ 4,757                        | \$ 516        |
| Restricted stock awards | 21                               | 18            | 76                              | 54            |
|                         | <u>\$ 2,516</u>                  | <u>\$ 185</u> | <u>\$ 4,833</u>                 | <u>\$ 570</u> |

Total compensation cost recognized for all stock-based compensation awards in the consolidated statements of operations and comprehensive loss is as follows:

|                            | Three months ended September 30, |               | Nine months ended September 30, |               |
|----------------------------|----------------------------------|---------------|---------------------------------|---------------|
|                            | 2013                             | 2012          | 2013                            | 2012          |
| Research and development   | \$ 1,683                         | \$ 85         | \$ 2,942                        | \$ 308        |
| General and administrative | 833                              | 100           | 1,891                           | 262           |
|                            | <u>\$ 2,516</u>                  | <u>\$ 185</u> | <u>\$ 4,833</u>                 | <u>\$ 570</u> |

As of September 30, 2013, there was \$10,379 of unrecognized compensation expense related to unvested stock options and restricted stock awards that is expected to be recognized over a weighted-average period of 2.9 years.

**Restricted Common Stock**

|  | Shares    | Weighted-average grant date fair value |
|--|-----------|--|
| Unvested balance at December 31, 2012  | 155       | \$ 0.95                                |
| Granted                                | —         | —                                      |
| Vested                                 | (66)      | 1.01                                   |
| Forfeited                              | —         | —                                      |
| Unvested balance at September 30, 2013 | <u>89</u> | <u>\$ 0.95</u>                         |

**Stock options**

The following table summarizes the stock option activity under the Company's equity award plans:

|   | Shares | Weighted-average exercise price per share |
|---|--------|---|
| Outstanding at December 31, 2012                  | 2,201  | \$ 2.09                                   |
| Granted   | 1,855  | \$ 7.12                                   |
| Exercised   | (37)   | \$ 2.46                                   |
| Canceled or forfeited                             | (42)   | \$ 3.79                                   |
| Outstanding at September 30, 2013                 | 3,977  | \$ 4.41                                   |
| Exercisable at September 30, 2013                 | 1,336  | \$ 2.06                                   |
| Vested and expected to vest at September 30, 2013 | 3,977  | \$ 4.41                                   |

There were 37 options exercised for the nine months ended September 30, 2013, resulting in total proceeds of \$92. In accordance with Company policy, the shares were issued from a pool of shares reserved for issuance under the stock plans described above.

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***Note receivable***

In November 2010, the Company received a non-recourse note from its Chief Executive Officer (“CEO”) in exchange for the purchase of 329 shares of restricted stock. Interest accrued on the note on an annual basis at a rate of four percent. In May 2013, prior to the initial filing of the registration statement in connection with the Company’s IPO, the CEO repaid the note in full plus all accrued interest. The Company recorded stock-based compensation expense in connection with this restricted stock award of \$16 and \$47 for the three and nine months ended September 30, 2013, respectively, and \$16 and \$47 for the three and nine months ended September 30, 2012, respectively.

***Employee Stock Purchase Plan***

On June 3, 2013, the Company’s board of directors adopted its 2013 Employee Stock Purchase Plan (“2013 ESPP”), which was subsequently approved by its stockholders and became effective upon the closing of the Company’s initial public offering on June 24, 2013. The 2013 ESPP authorizes the initial issuance of up to a total of 238 shares of the Company’s common stock to participating employees. Unless otherwise determined by the administrator of the 2013 ESPP, the first offering will begin on January 1 of the year designated by the administrator and end on the following June 30.

**9. Income taxes**

Deferred tax assets and deferred tax liabilities are recognized based on temporary differences between the financial reporting and tax basis of assets and liabilities using statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized. There were no significant income tax provisions or benefits for the three or nine months ended September 30, 2013 and 2012. Due to the uncertainty surrounding the realization of the favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company’s otherwise recognizable net deferred tax assets.

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## 10. Net loss per share

The following table sets forth the computation of the Company's basic and diluted net (loss) income per share attributable to common stockholders:

|  | Three months ended<br>September 30, |                | Nine months ended<br>September 30, |                |
|--|-------------------------------------|----------------|------------------------------------|----------------|
|  | 2013                                | 2012           | 2013                               | 2012           |
| <b>Numerator:</b>  |                                     |                |                                    |                |
| Net loss   | \$ (6,113)                          | \$ (5,041)     | \$ (17,240)                        | \$ (14,672)    |
| Accretion and dividends on convertible preferred stock   | —                                   | (487)          | —                                  | (3,057)        |
| Gain on extinguishment of convertible preferred stock  | —                                   | 23,114         | —                                  | 23,114         |
| Net income attributable to participating securities  | —                                   | (17,271)       | —                                  | (5,274)        |
| Net (loss) income applicable to common stockholders - basic  | <u>\$ (6,113)</u>                   | <u>\$ 315</u>  | <u>\$ (17,240)</u>                 | <u>\$ 111</u>  |
| Net loss   | \$ (6,113)                          | \$ (5,041)     | \$ (17,240)                        | \$ (14,672)    |
| Accretion and dividends on convertible preferred stock   | —                                   | (487)          | —                                  | (3,057)        |
| Gain on extinguishment of convertible preferred stock  | —                                   | 23,114         | —                                  | 23,114         |
| Net income applicable to participating securities  | —                                   | (16,954)       | —                                  | (5,142)        |
| Net (loss) income applicable to common stockholders - diluted  | <u>\$ (6,113)</u>                   | <u>\$ 632</u>  | <u>\$ (17,240)</u>                 | <u>\$ 243</u>  |
| <b>Denominator:</b>  |                                     |                |                                    |                |
| Weighted-average common shares outstanding - basic   | 23,623                              | 275            | 8,786                              | 250            |
| Weighted-average restricted shares outstanding   | —                                   | 184            | —                                  | 206            |
| Weighted-average number of common shares issuable upon exercise of outstanding warrants, based on treasury stock method              | —                                   | 95             | —                                  | 95             |
| Weighted-average number of common shares used in computing net (loss) income per share attributable to common stockholders - diluted | <u>23,623</u>                       | <u>554</u>     | <u>8,786</u>                       | <u>551</u>     |
| Net (loss) income per share applicable to common stockholders - basic  | <u>\$ (0.26)</u>                    | <u>\$ 1.15</u> | <u>\$ (1.96)</u>                   | <u>\$ 0.44</u> |
| Net (loss) income per share applicable to common stockholders - diluted  | <u>\$ (0.26)</u>                    | <u>\$ 1.14</u> | <u>\$ (1.96)</u>                   | <u>\$ 0.44</u> |

The following common stock equivalents were excluded from the calculation of diluted net (loss) income per share for the periods indicated because including them would have had an anti-dilutive effect:

|                           | Three months ended September 30, |            | Nine months ended September 30, |            |
|---------------------------|----------------------------------|------------|---------------------------------|------------|
|                           | 2013                             | 2012       | 2013                            | 2012       |
| Warrants                  | 440                              | 338        | 440                             | 338        |
| Outstanding stock options | 3,977                            | 291        | 3,977                           | 291        |
| Unvested restricted stock | 89                               | —          | 89                              | —          |
|                           | <u>4,506</u>                     | <u>629</u> | <u>4,506</u>                    | <u>629</u> |

## 11. Subsequent events

The Company has evaluated all events or transactions that occurred after September 30, 2013. In the judgment of management, there were no material events that impacted the unaudited condensed consolidated financial statements or disclosures.

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

*The following information should be read in conjunction with the unaudited financial information and the notes thereto included in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in the Prospectus that forms a part of our Registration Statement on Form S-1 (File No. 333-188605), which was filed with the Securities and Exchange Commission (the "SEC") pursuant to Rule 424 on June 19, 2013 (the "Prospectus").*

*Except for the historical information contained herein, the matters discussed in this Quarterly Report on Form 10-Q may be deemed to be forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. In this Quarterly Report on Form 10-Q, words such as "may," "will," "expect," "anticipate," "estimate," "intend," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements.*

*Our actual results and the timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, they may not be predictive of results or developments in future periods.*

*The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report on Form 10-Q, including those risks identified under Part II, Item 1A. Risk Factors.*

*We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.*

### **Overview**

We are a clinical-stage biotechnology company focused on transforming the lives of patients with severe genetic and orphan diseases using gene therapy. We believe that gene therapy has the potential to change the way these patients are treated by correcting the underlying genetic defect that is the cause of their disease, rather than offering solutions that only address their symptoms. We and our scientific collaborators have generated what we believe is human proof-of-concept data for our gene therapy platform in two underserved diseases, each of which has been granted orphan drug status by U.S. and European regulatory authorities. We have initiated a Phase II/III clinical study of our most advanced product candidate, Lenti-D, to evaluate its safety and efficacy in subjects with childhood cerebral adrenoleukodystrophy, or CCALD, a rare, hereditary neurological disorder affecting young boys that is often fatal. In October 2013, we announced that the first subject had been treated in this study. We also have initiated Phase I/II clinical studies in both the United States and Europe of our next most advanced product candidate, LentiGlobin, to evaluate its safety and efficacy in subjects with  $\beta$ -thalassemia major and, in the European clinical study, sickle cell disease, or SCD, which are hereditary blood disorders that often lead to severe anemia and shortened lifespans. In addition, in March 2013, we announced a global strategic collaboration with Celgene Corporation to discover, develop and commercialize novel, disease-altering gene therapies in oncology.

Since our inception in 1992, we have devoted substantially all of our resources to our development efforts relating to our product candidates, including activities to manufacture product in compliance with good manufacturing practices, or GMP, preparing to conduct clinical studies of our product candidates, providing general and administrative support for these operations and protecting our intellectual property. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through the private placement of preferred stock, common stock, convertible notes and warrants to purchase common stock. In March 2013, we entered into a strategic collaboration with Celgene Corporation, or Celgene, to discover, develop and commercialize novel, disease-altering gene therapies in oncology. This collaboration has an initial term of three years, and Celgene has made a \$75.0 million up-front, non-refundable cash payment to us as consideration for entering into the collaboration. During the three months and nine months ended September 30, 2013, we recognized \$6.3 million and \$13.5 million, respectively, of revenue associated with our collaboration with Celgene related to the research and development services performed. As of September 30, 2013, there is \$61.5 million of deferred revenue related to our collaboration with Celgene that is classified as current or long-term in the accompanying balance sheet based on the contractual term of the arrangement.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were \$17.2 million for the nine months ended September 30, 2013, and our accumulated deficit was \$90.6 million as of September 30, 2013. Substantially all our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect our expenses will increase substantially in connection with our ongoing and planned activities, as we:

- conduct clinical studies for our Lenti-D and LentiGlobin product candidates;

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- continue our research and development efforts;
- increase research and development related activities for the discovery and development of oncology products;
- develop product candidates in connection with our recently-announced strategic collaboration with Celgene;
- manufacture clinical study materials and develop large-scale manufacturing capabilities;
- seek regulatory approval for our product candidates;
- add personnel to support our product development and commercialization efforts; and
- operate as a public company.

We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years and is subject to significant uncertainty. We have no commercial-scale manufacturing facilities, and all of our manufacturing activities are contracted out to third parties. Additionally, we currently utilize third-party contract research organizations, or CROs, to carry out our clinical development activities; and we do not yet have a sales organization. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing, and distribution. Accordingly, we will seek to fund our operations through public or private equity or debt financings or other sources. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our products.

On June 3, 2013, our board of directors and our stockholders approved a one-for-18.967 reverse stock split of our outstanding common stock, which was effected on June 3, 2013. Stockholders entitled to fractional shares as a result of the reverse stock split will receive a cash payment in lieu of receiving fractional shares. Our historical share and per share information have been retroactively adjusted to give effect to this reverse stock split. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices, if applicable, were proportionately increased in accordance with the terms of the agreements governing such securities. Shares of common stock reserved for issuance upon the conversion of our Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock were proportionately reduced; and the respective conversion prices were proportionately increased.

On June 24, 2013, we completed our initial public offering, or IPO, whereby we sold 6,832,352 shares of common stock (inclusive of 891,176 shares of common stock sold by us pursuant to the full exercise of an overallotment option granted to the underwriters in connection with the offering) at a price of \$17.00 per share. The shares began trading on the Nasdaq Global Select Market on June 19, 2013. The aggregate net proceeds received by us from the IPO were \$104.9 million, net of underwriting discounts and commissions and estimated offering expenses payable by us. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 16,388,510 shares of common stock; and warrants exercisable for convertible preferred stock were automatically converted into warrants exercisable for 337,952 shares of common stock, resulting in the reclassification of the related convertible preferred stock warrant liability of approximately \$0.7 million to additional paid-in capital. Additionally, we are now authorized to issue 125,000,000 shares of common stock and 5,000,000 shares of preferred stock.

## **Financial operations overview**

### ***Revenue***

To date, we have not generated any revenues from the sale of products. Our revenues have been derived from collaboration arrangements, research fees, license fees, and grant revenues.

Collaboration revenue is generated exclusively from our collaboration arrangement with Celgene. The terms of this arrangement contain multiple deliverables, which include at inception: (i) discovery, research and development services, (ii) participation on the joint steering committee and (iii) participation on the patent committee. We recognize arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 605, *Revenue Recognition*, or ASC 605, are satisfied for that particular unit of accounting.

Revenue from the Celgene arrangement associated with discovery, research and development services, joint steering committee services and patent committee services is recognized ratably over the associated period of performance, which is initially three years.

Research and license fee revenue is primarily generated through license and research and development agreements with strategic partners and nonprofit organizations for the development and commercialization of our product candidates. There are no performance, cancellation, termination, or refund provisions in any of our arrangements that contain material financial consequences to us.

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Nonrefundable license fees are recognized as revenue upon delivery provided there are no undelivered elements in the arrangement. Research fees are recognized as revenue over the period we perform the associated services or on a straight-line basis if the pattern of performance cannot be estimated.

Our ability to generate product revenue and become profitable depends upon our ability to successfully commercialize products. We expect to incur losses for the foreseeable future, and we expect these losses to increase as we continue our development of, and seek regulatory approvals for, our product candidates and begin to commercialize any approved products. Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate revenues from the sale of our products, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

### ***Research and development expenses***

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with CROs and investigative sites that will conduct our clinical studies;
- costs of acquiring, developing, and manufacturing clinical study materials;
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supplies; and
- costs associated with preclinical activities and regulatory operations.

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites. We cannot determine with certainty the duration and completion costs of the current or future clinical studies of our product candidates or if, when, or to what extent we will generate revenues from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs, and timing of clinical studies and development of our product candidates will depend on a variety of factors, including:

- the scope, rate of progress, and expense of our ongoing as well as any additional clinical studies and other research and development activities;
- future clinical study results;
- uncertainties in clinical study enrollment rate;
- significant and changing government regulation; and
- the timing and receipt of any regulatory approvals.

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

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From inception through September 30, 2013, we have incurred \$85.7 million in research and development expenses. We plan to increase our research and development expenses for the foreseeable future as we continue the development of our Lenti-D and LentiGlobin product candidates and conduct research and development activities under our strategic collaboration with Celgene. Our research and development activities include the following:

- We have initiated a Phase II/III clinical study to examine the feasibility, safety and efficacy of our Lenti-D product candidate in the treatment of CCALD. In October 2013, we announced that the first subject had been treated in this study.
- We have initiated a Phase I/II clinical study in France to study the feasibility, safety and efficacy of our LentiGlobin product candidate in the treatment of subjects with  $\beta$ -thalassemia major and severe SCD.
- We have initiated a Phase I/II clinical study in the United States to study the feasibility, safety and efficacy of our LentiGlobin product candidate in the treatment of subjects with  $\beta$ -thalassemia major.
- We will continue to manufacture clinical study materials in support of our clinical studies.

Our direct research and development expenses consist principally of external costs, such as start-up fees paid to investigators, consultants, central laboratories and CROs in connection with our clinical studies, and costs related to acquiring and manufacturing clinical study materials. We do not allocate personnel-related costs, costs associated with our general platform improvements, depreciation or other indirect costs to specific programs, as they are deployed across multiple projects under development and, as such, are separately classified as personnel and other expenses in the table below:

|   | Three months ended September 30, |                 | Nine months ended September 30, |                  |
|---|----------------------------------|-----------------|---------------------------------|------------------|
|   | 2013                             | 2012            | 2013                            | 2012             |
|   | (in thousands)                   |                 |                                 |                  |
| Lenti-D                                       | \$ 616                           | \$ 784          | \$ 2,642                        | \$ 2,250         |
| LentiGlobin                                   | 2,519                            | 1,133           | 6,398                           | 2,866            |
| Pre-clinical programs                         | 226                              | —               | 322                             | —                |
| Total direct research and development expense | 3,361                            | 1,917           | 9,362                           | 5,116            |
| Employee- and contractor-related expenses     | 4,114                            | 1,194           | 9,085                           | 4,269            |
| Platform-related lab expenses                 | 151                              | 109             | 774                             | 517              |
| Facility expenses                             | 794                              | 146             | 1,415                           | 451              |
| Other expenses                                | 286                              | 135             | 601                             | 360              |
| Personnel and other expenses                  | 5,345                            | 1,584           | 11,875                          | 5,597            |
| Total research and development expense        | <u>\$ 8,706</u>                  | <u>\$ 3,501</u> | <u>\$ 21,237</u>                | <u>\$ 10,713</u> |

### General and administrative expenses

General and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation and travel expenses for our employees in executive, operational, finance and human resource functions. Other general and administrative expenses include facility-related costs and professional fees for directors, accounting and legal services and expenses associated with obtaining and maintaining patents.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our product candidates. We also anticipate increased expenses related to audit, legal, regulatory, and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance premiums, and investor relations costs associated with being a public company. Additionally, if and when we believe a regulatory approval of the first product candidate appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidates.

### Other income (expense), net

Other income and expense consists primarily of interest income earned on cash and cash equivalents, the re-measurement gain or loss associated with the change in the fair value of the preferred stock warrant liability, and foreign currency gain or loss. We use the Black-Scholes option pricing model to estimate the fair value of the warrants.

We base the estimates in the Black-Scholes option pricing model, in part, on subjective assumptions, including stock price volatility, risk-free interest rate, dividend yield, and the fair value of the preferred stock underlying the warrants. The re-measurement gain or loss associated with the change in the fair value of the preferred stock warrant liability each reporting period prior to becoming a public company is recognized as a component of other income (expense), net.

## Results of Operations

### Comparison of the three months ended September 30, 2013 and September 30, 2012:

|                             | Three months ended September 30, |            | Increase<br>(Decrease) |
|-----------------------------|----------------------------------|------------|------------------------|
|                             | 2013                             | 2012       |                        |
|                             | (in thousands)                   |            |                        |
| Revenue:                    |                                  |            |                        |
| Collaboration revenue       | \$ 6,251                         | \$ —       | \$ 6,251               |
| Research and license fees   | 134                              | 85         | 49                     |
| Total revenue               | 6,385                            | 85         | 6,300                  |
| Operating expenses:         |                                  |            |                        |
| Research and development    | 8,706                            | 3,501      | 5,205                  |
| General and administrative  | 3,836                            | 1,608      | 2,228                  |
| Total operating expenses    | 12,542                           | 5,109      | 7,433                  |
| Loss from operations        | (6,157)                          | (5,024)    | 1,133                  |
| Other income (expense), net | 44                               | (17)       | (61)                   |
| Net loss                    | \$ (6,113)                       | \$ (5,041) | \$ 1,072               |

**Revenue.** Total revenue was \$6.4 million for the three months ended September 30, 2013, compared to \$0.1 million for the three months ended September 30, 2012. The increase of \$6.3 million was primarily due to the Celgene collaboration. In the three months ended September 30, 2013, we recorded \$6.3 million in recognition of amounts allocated to research and development services from the Celgene collaboration, which was entered into in March 2013 and is expected to be recognized on a straight-line basis through March 2016, and \$0.1 million of research and license fees.

**Research and development expenses.** Research and development expenses were \$8.7 million for the three months ended September 30, 2013, compared to \$3.5 million for the three months ended September 30, 2012. The increase of \$5.2 million was primarily due to the increase in headcount and clinical trial related expenses to support the advancement of our programs, including the new Celgene collaboration, and included the following increases in expenses:

- Direct research and development expenses:
  - \$0.8 million of materials production costs in preparation for and upon initiation of the ALD 102, HGB-204 and HGB-205 clinical studies.
  - \$0.6 million of clinical trial related costs related to initiation of clinical studies in 2013.
  - \$0.3 million of direct project lab supplies related to increased headcount and scale up process development activities.
- Personnel and other expenses:
  - \$2.7 million of employee compensation and benefits to support increased development activities related to the three clinical studies initiated and in support of preclinical programs in 2013. The increased headcount resulted in an incremental \$0.1 million of recruiting and \$0.1 million of travel expense.
  - \$0.6 million in facility-related expenses to accommodate increased lab headcount.
  - \$0.1 million in accelerated depreciation due to the shortened expected useful life of assets relating to our current building lease.

**General and administrative expenses.** General and administrative expenses were \$3.8 million for the three months ended September 30, 2013, compared to \$1.6 million for the three months ended September 30, 2012. The increase of \$2.2 million was primarily due to the following increases in expenses: \$1.3 million of employee-related costs to support our overall growth; \$0.1 million of contractors and consultants expenses and \$0.4 million of professional fees to support the requirements of being a public company; \$0.1 million in general office expenses as a result of increased headcount; and \$0.1 million in accelerated depreciation due to the shortened expected useful life of assets relating to the current building lease.

**Other income (expense), net.** Other income (expense), net, was \$0.04 million for the three months ended September 30, 2013, compared to \$(0.02) million for the three months ended September 30, 2012. The decrease of \$0.06 million was primarily due to the re-measurement of our convertible preferred stock warrants and foreign currency gain.



**Comparison of the nine months ended September 30, 2013 and September 30, 2012:**

|                             | <u>Nine months ended September 30,</u> |                    | <u>Increase<br/>(Decrease)</u> |
|-----------------------------|--|--------------------|--------------------------------|
|                             | <u>2013</u>                            | <u>2012</u>        |                                |
|                             | (in thousands)                         |                    |                                |
| Revenue:                    |  |                    |                                |
| Collaboration revenue       | \$ 13,542                              | \$ —               | \$ 13,542                      |
| Research and license fees   | 304                                    | 255                | 49                             |
| Total revenue               | 13,846                                 | 255                | 13,591                         |
| Operating expenses:         |  |                    |                                |
| Research and development    | 21,237                                 | 10,713             | 10,524                         |
| General and administrative  | 9,441                                  | 4,302              | 5,139                          |
| Total operating expenses    | 30,678                                 | 15,015             | 15,663                         |
| Loss from operations        | (16,832)                               | (14,760)           | 2,072                          |
| Other income (expense), net | (408)                                  | 88                 | 496                            |
| Net loss                    | <u>\$ (17,240)</u>                     | <u>\$ (14,672)</u> | <u>\$ 2,568</u>                |

**Revenue.** Total revenue was \$13.8 million for the nine months ended September 30, 2013, compared to \$0.3 million for the nine months ended September 30, 2012. The increase of \$13.6 million was primarily due to the Celgene collaboration. In the nine months ended September 30, 2013, we recorded \$13.5 million in recognition of amounts allocated to research and development services from the Celgene collaboration, which was entered into in March 2013 and is expected to be recognized on a straight-line basis through March 2016, and \$0.3 million of research and license fees.

**Research and development expenses.** Research and development expenses were \$21.2 million for the nine months ended September 30, 2013, compared to \$10.7 million for the nine months ended September 30, 2012. The increase of \$10.5 million was primarily due to the increase in headcount and clinical trial related expenses to support the advancement of our programs, including the new Celgene collaboration, and included the following increases in expenses:

- Direct research and development expenses:
  - \$1.4 million of clinical trial related costs related to initiation of the ALD 102, HGB-204 and HGB-205 clinical studies.
  - \$0.8 million of costs for clinical and regulatory consultants to support regulatory filing and other clinical start-up activities.
  - \$1.1 million of materials production costs and non-clinical services (e.g. site audits) in preparation for and upon initiation of the three clinical studies initiated in 2013.
  - \$0.8 million of direct project lab supplies related to increased headcount and scale up process development activities.
- Personnel and other expenses:
  - \$4.5 million of employee compensation and benefits, to support increased development activities related to the three clinical studies initiated and in support of preclinical programs in 2013. The increased headcount resulted in an incremental \$0.2 million of recruiting costs and \$0.3 million of travel expense.
  - \$0.3 million of lab supplies related to increased headcount and increased scale up process development activities.
  - \$1.0 million in facility related expenses to accommodate increased lab headcount.

**General and administrative expenses.** General and administrative expenses were \$9.4 million for the nine months ended September 30, 2013, compared to \$4.3 million for the nine months ended September 30, 2012. The increase of \$5.1 million was primarily due to \$2.9 million of employee-related costs to support our overall growth; \$0.9 million of contractor and consultant expenses incurred in connection with the preparation of the recent IPO; and \$0.7 million of professional fees to support the requirements of being a public company.

**Other income (expense), net.** Other income (expense), net, was \$(0.4) million for the nine months ended September 30, 2013, compared to \$0.1 million for the nine months ended September 30, 2012. The increase of \$(0.5) million was primarily due to the re-measurement of our convertible preferred stock warrants.

## Liquidity and Capital Resources

We have incurred losses and cumulative negative cash flows from operations since our inception in April 1992, and as of September 30, 2013, we had an accumulated deficit of \$90.6 million. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

We have funded our operations principally from the sale of common stock, preferred stock, convertible notes and warrants to purchase common stock. In March 2013, we entered into a strategic collaboration with Celgene to discover, develop and commercialize novel, disease-altering gene therapies in oncology. This collaboration has an initial term of three years, and Celgene has made a \$75.0 million up-front, non-refundable cash payment to us as consideration for entering into the collaboration. In June 2013, we completed our IPO, which resulted in aggregate net proceeds to us of \$104.9 million. As of September 30, 2013, we had cash and cash equivalents of approximately \$216.8 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Currently, our funds are held in money market mutual funds consisting of U.S. government-backed securities.

### Sources of Liquidity

#### Cash Flows

The following table sets forth the primary sources and uses of cash for each of the periods below:

|   | Nine months ended September 30, |             |
|---|---------------------------------|-------------|
|   | 2013                            | 2012        |
|   | (in thousands)                  |             |
| Net cash provided by (used in):           |                                 |             |
| Operating activities                      | \$ 48,905                       | \$ (14,408) |
| Investing activities                      | (4,533)                         | 2,955       |
| Financing activities                      | 105,408                         | 59,867      |
| Net increase in cash and cash equivalents | \$ 149,780                      | \$ 48,414   |

*Cash Flows from Operating Activities.* The significant increase in cash provided by operating activities for the nine months ended September 30, 2013, compared to the nine months ended September 30, 2012, is primarily due to the up-front payment related to the Celgene collaboration agreement that we received in March 2013. The net cash provided by operating activities was \$48.9 million for the nine months ended September 30, 2013 and primarily consisted of a net loss of \$17.2 million adjusted for non-cash items including stock-based compensation of \$4.8 million, depreciation and amortization of \$0.6 million, re-measurement of warrants of \$0.4 million and a net increase in operating assets and liabilities of \$60.3 million. The significant items in the increase in operating assets and liabilities include an increase in deferred revenue of \$61.2 million due to the up-front payment related to the Celgene collaboration and an increase in accrued expenses and other current liabilities of \$4.0 million, slightly offset by an increase in prepaid expenses and other current assets of \$3.9 million and a decrease in accounts payable of \$1.0 million.

*Cash Flows from Investing Activities.* The net cash used in investing activities was \$4.5 million for the nine months ended September 30, 2013, and was primarily due to the purchase of fixed assets of \$3.4 million and the new \$1.2 million cash-collateralized irrevocable standby letter of credit on the new building lease that we signed in June 2013. The fixed asset purchases primarily consisted of purchases of lab equipment for the additional lab space added during the first quarter of 2013 and lab equipment to support the start-up of the Celgene program. The new \$1.3 million letter of credit, naming the landlord as beneficiary, is reduced to \$1.0 million, \$0.8 million, and \$0.6 million upon the rent commencement date and the first and second anniversaries of the rent commencement date, respectively.

*Cash Flows from Financing Activities.* The net cash provided by financing activities was \$105.4 million for the nine months ended September 30, 2013 and was primarily due to the issuance of 6,832,352 common stock related to our initial public offering that closed on June 24, 2013, for total proceeds of \$105.0 million, net of \$11.2 million in issuance costs paid, the repayment of the non-recourse note collateralized by restricted stock of \$0.3 million, and proceeds from the exercise of common stock options of \$0.1 million.

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***Operating Capital Requirements***

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that we will continue to generate losses for the foreseeable future; and we expect the losses to increase as we continue the development of and seek regulatory approvals for our product candidates and begin to commercialize any approved products. We are subject to all of the risks incident in the development of new gene therapy products; and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. We expect to incur additional costs associated with operating as a public company. We anticipate that we will need substantial additional funding in connection with our continuing operations. We believe that our existing cash and cash equivalents as of September 30, 2013 will be sufficient to fund our projected operating requirements through at least the end of 2015. However, we may require additional capital for the further development of our existing product candidates and may also need to raise additional funds sooner to pursue other development activities related to additional product candidates.

Until we can generate a sufficient amount of revenue from our products, if ever, we expect to finance future cash needs through public or private equity or debt offerings. Additional capital may not be available on reasonable terms, if at all. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates. If we raise additional funds through the issuance of additional debt or equity securities, it could result in dilution to our existing stockholders, increased fixed payment obligations and these securities may have rights senior to those of our common stock. If we incur indebtedness, we could become subject to covenants that would restrict our operations and potentially impair our competitiveness, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Any of these events could significantly harm our business, financial condition and prospects.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties; and actual results could vary as a result of a number of factors. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of clinical studies for our products, including our Phase II/III Lenti-D study and our Phase I/II LentiGlobin studies;
- the outcome, timing and cost of regulatory approvals by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies than those that we currently expect;
- the ability of our product candidates to progress through clinical development successfully;
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights;
- our need to expand our research and development activities;
- our need and ability to hire additional personnel;
- our need to implement additional infrastructure and internal systems;
- the effect of competing technological and market developments; and
- the cost of establishing sales, marketing and distribution capabilities for any products for which we may receive regulatory approval.

If we cannot expand our operations or otherwise capitalize on our business opportunities because we lack sufficient capital, our business, financial condition and results of operations could be materially adversely affected.

***Contractual Obligations and Commitments***

There have been no material changes to our contractual obligations from those described in our Prospectus that forms a part of our Registration Statement on Form S-1 (File No. 333-188605), which was filed with the Securities and Exchange Commission, or the SEC, pursuant to Rule 424 on June 19, 2013, except as noted below:

On June 3, 2013, we entered into a new nine-year building lease for approximately 43,600 square feet of space at 150 Second Street, Cambridge, Massachusetts, commencing on the earlier of the substantial completion of the build-out work or January 1, 2014. The total operating lease obligation for this lease is \$24.2 million, of which \$0.6 is payable in less than one year; \$5.2 million in one to three years; \$5.5 million in three to five years; and the balance of \$12.9 million is payable in more than five years from September 30, 2013.

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**Off-Balance Sheet Arrangements**

As of September 30, 2013, we did not have any off-balance sheet arrangements as defined in the rules and regulations of the Securities and Exchange Commission.

**Critical Accounting Policies**

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued research and development expenses and stock-based compensation. We base our estimates on historical experience, known trends and events and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies.

There have been no material changes to our critical accounting policies from those described in "Management's discussion and analysis of financial condition and results of operations" included in our Prospectus included in the Registration Statement on Form S-1 (File No. 333-188605), which was filed with the Securities and Exchange Commission pursuant to Rule 424 on June 19, 2013.

**Item 3. Quantitative and Qualitative Disclosures about Market Risks**

We are exposed to market risk related to changes in interest rates. As of September 30, 2013 and December 31, 2012, we had cash and cash equivalents of \$216.8 million and \$67.0 million, respectively, primarily money market mutual funds consisting of U.S. government-backed securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

**Item 4. Controls and Procedures**

***Management's Evaluation of our Disclosure Controls and Procedures***

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities and Exchange Act of 1934 is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of September 30, 2013, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities and Exchange Act of 1934). Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2013, our disclosure controls and procedures were effective at the reasonable assurance level.

***Changes in Internal Control Over Financial Reporting***

During the quarter ended September 30, 2013, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II. OTHER INFORMATION

### Item 1. Legal Proceedings

In the ordinary course of business, we are from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements and other matters. While the outcome of these proceedings and claims cannot be predicted with certainty, as of September 30, 2013, we were not party to any legal or arbitration proceedings that may have, or have had in the recent past, significant effects on our financial position or profitability. No governmental proceedings are pending or, to our knowledge, contemplated against us. We are not a party to any material proceedings in which any director, member of senior management or affiliate of ours is either a party adverse to us or our subsidiaries or has a material interest adverse to us or our subsidiaries.

### Item 1A. Risk Factors

*An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our financial statements and related notes hereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.*

#### Risks related to our financial condition and capital requirements

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

We are a clinical-stage biotechnology company, and we have not yet generated significant revenues. We have incurred net losses in each year since our inception in 1992, including net losses of \$15.6 million and \$23.7 million for the years ended December 31, 2011 and 2012, respectively, and \$17.2 million for the nine months ended September 30, 2013. As of September 30, 2013, we had an accumulated deficit of \$90.6 million.

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

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

- continue our research and preclinical and clinical development of our product candidates;
- expand the scope of our current clinical studies for our product candidates;
- initiate additional preclinical, clinical or other studies for our product candidates, including under our collaboration agreement with Celgene Corporation;
- further develop the manufacturing process for our vectors or our product candidates;
- change or add additional manufacturers or suppliers;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical studies;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- seek to identify and validate additional product candidates;
- acquire or in-license other product candidates and technologies;
- make milestone or other payments under any in-license agreements;
- maintain, protect and expand our intellectual property portfolio;
- attract and retain skilled personnel;

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- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with any of the above.

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

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

Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize our product candidates. We do not anticipate generating revenues from product sales for the foreseeable future, if ever. Our ability to generate future revenues from product sales depends heavily on our success in:

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

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

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

We are currently advancing our Lenti-D and LentiGlobin product candidates through clinical development and other product candidates through preclinical development. Developing gene therapy products is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates in clinical studies.

As of September 30, 2013, our cash and cash equivalents were \$216.8 million. We expect that our existing cash and cash equivalents will be sufficient to fund our current operations through at least the end of 2015. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

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Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

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

### **Risks related to the discovery and development of our product candidates**

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

We have concentrated our therapeutic product research and development efforts on our gene therapy platform, and our future success depends on the successful development of this therapeutic approach. There can be no assurance that any development problems we experience in the future related to our gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the EMA 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 and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. At the moment, only one gene therapy product, UniQure's Glybera, which received marketing authorization from the EMA in 2012, has been approved in the Western world, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either Europe or the United States. Approvals by the EMA may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene and cell therapy products have evolved and may continue to change in the future. For example, the FDA has established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical studies conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical study, even if the FDA has reviewed the study and approved its initiation. Conversely, the FDA can put an investigational new drug application, or IND, on clinical hold even if the RAC has provided a favorable review. Also, before a clinical study can begin at an NIH-funded institution, that institution's institutional review board, or IRB, and its Institutional Biosafety Committee will have to review the proposed clinical study to assess the safety of the study. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

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



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

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends on the speed at which we can recruit patients to participate in testing our product candidates. We have experienced delays in some of our clinical studies, and we may experience similar delays in the future. If patients are unwilling to participate in our gene therapy studies because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical studies for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of potential products may 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 studies altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete our clinical studies in a timely manner. Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the study protocol;
- size of the patient population;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;
- proximity and availability of clinical study sites for prospective patients;
- availability of competing therapies and clinical studies;
- efforts to facilitate timely enrollment in clinical studies;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

In particular, each of the conditions for which we plan to evaluate our current product candidates are rare genetic disorders with limited patient pools from which to draw for clinical studies. It has been estimated that about 1.5% (80 to 90 million people) of the global population are carriers of  $\beta$ -thalassemia, with about 60,000 symptomatic individuals born annually, the great majority in the developing world. According to Thalassemia International Federation, about 288,000 patients with  $\beta$ -thalassemia major are alive and registered as receiving regular treatment around the world, of which it is estimated that about 15,000 live in the United States and Europe. The global incidence of SCD is estimated to be 250,000-300,000 births annually with a global prevalence estimated to be about 20-25 million. The worldwide incidence rate for adrenoleukodystrophy, or ALD, the superset of CCALD, is approximately one in 20,000 newborn males. CCALD accounts for about 30-40% of patients diagnosed with ALD. Further, because newborn screening for CCALD is not widely adopted, and it can be difficult to diagnose CCALD in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our study. The eligibility criteria of our clinical studies will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. Finally, our treatment process requires that the patient be near one of our transduction facilities, as the hematopoietic stem cells, or HSCs, have limited viability following harvest and cannot be transported long distances.

Our current product candidates are being developed to treat rare conditions. We plan to seek initial marketing approval in the United States and Europe. We may not be able to initiate or continue clinical studies if we cannot enroll a sufficient number of eligible patients to participate in the clinical studies required by the FDA or the EMA or other regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical study in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

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

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



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

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

- delays in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical study sites;
- delays in obtaining required Institutional Review Board, or IRB, or Institutional Ethics Committee approval at each clinical study site;
- delays in recruiting suitable patients to participate in our clinical studies;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical study operations or study sites;
- failure by our CROs, other third parties or us to adhere to clinical study requirements;
- failure to perform in accordance with the FDA's good clinical practices, or GCP, or applicable regulatory requirements in other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;
- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical study sites or patients dropping out of a study;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical study delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

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

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes with the way the product is administered;
- be required to perform additional clinical studies to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Treatment with our product candidates involves chemotherapy and myeloablative treatments, which can cause side effects or adverse events that are unrelated to our product candidate, but may still impact the success of our clinical studies. Additionally, our product candidates could potentially cause other adverse events that have not yet been predicted. The inclusion of critically ill patients in our clinical studies may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using. As described above, any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our products.

***We have not tested any of our current viral vectors or product candidates derived from these viral vectors in clinical studies. Success in early clinical studies may not be indicative of results obtained in later studies.***

Neither our current viral vectors nor our product candidates have ever been evaluated in human clinical studies, and we may experience unexpected results in the future. Earlier gene therapy clinical studies, which we believe serve as proof-of-concept for our product candidates, utilized lentiviral vectors similar to ours. However, these studies should not be relied upon as evidence that our future clinical studies will succeed. Study designs and results from previous studies are not necessarily predictive of our future clinical study designs or results, and initial results may not be confirmed upon full analysis of the complete study data. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical studies.

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

***The results from our ALD-102 Study may not be sufficiently robust to support the submission of marketing approval for our Lenti-D product candidate. Before we submit Lenti-D for marketing approval, the FDA and the EMA may require us to enroll additional subjects, conduct additional clinical studies, or evaluate subjects for an additional follow-up period.***

The FDA has advised us that our ALD-102 Study, which is a single-arm, open-label study to evaluate the safety and efficacy of our Lenti-D product candidate to halt the progression of CCALD, may not be deemed to be a pivotal study or may not provide sufficient support for a Biologics License Application, or BLA, submission. The FDA normally requires two pivotal clinical studies to approve a drug or biologic product, and thus the FDA may require that we conduct additional clinical studies of Lenti-D prior to a BLA submission. The FDA typically does not consider a single clinical study to be adequate to serve as a pivotal study unless it is, among other things, well-controlled and demonstrates a clinically meaningful effect on mortality, irreversible morbidity, or prevention of a disease with potentially serious outcome, and a confirmatory study would be practically or ethically impossible. Due to the nature of CCALD and the limited number of patients with this condition, a placebo-controlled and blinded study is not practicable for ethical and other reasons. However, it is still possible that, even if we achieve favorable results in the ALD-102 Study, the FDA may require us to enroll additional subjects or conduct additional clinical studies, possibly involving a larger sample size or a different clinical study design, particularly if the FDA does not find the results from the ALD-102 Study to be sufficiently persuasive to support a BLA submission. The FDA may also require that we conduct a longer follow-up period of subjects treated with our Lenti-D product candidate prior to accepting our BLA submission.

In addition, the ALD-102 Study was not designed to achieve a statistically significant efficacy determination. Rather, we expect that safety and efficacy will be evaluated in light of the data collected in our retrospective data collection study, the ALD-101 Study. However, due to the nature of this retrospective data collection study, and the limited number of patients with this condition, the FDA has advised us that the ALD-101 Study is not sufficiently robust to serve as a conventional historical control group and as a basis of comparison against the results of the ALD-102 Study. Thus, we expect that the FDA will assess the totality of the safety and efficacy data from our ALD-102 Study in reviewing any future BLA submission for our Lenti-D product candidate. Based on this assessment, the FDA may require that we conduct additional preclinical or clinical studies prior to submitting or approving a BLA for this indication.

It is possible that the FDA or the EMA may not consider the results of this study to be sufficient for approval of Lenti-D for this indication. If the FDA or the EMA requires additional studies, we would incur increased costs and delays in the marketing approval process, which may require us to expend more resources than we have available. In addition, it is possible that the FDA and the EMA may have divergent opinions on the elements necessary for a successful BLA and Marketing Authorization Application, or MAA, respectively, which may cause us to alter our development, regulatory and/or commercialization strategies.

***In previous clinical studies involving viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of leukemia due to vector-related insertional oncogenesis. If our vectors demonstrate a similar effect, we may be required to halt or delay further clinical development of our product candidates.***

A significant risk in any gene therapy product based on viral vectors is that the vector will insert near cancer-causing oncogenes leading to uncontrolled clonal proliferation of mature cancer cells in the patient. For example, in 2003, 20 subjects treated for X-linked severe combined immunodeficiency in two gene therapy studies using a murine gamma-retroviral vector showed correction of the disease, but the studies were terminated after five subjects developed leukemia (four of whom were subsequently cured). The cause of these adverse events was shown to be insertional oncogenesis, which is the process whereby the corrected gene inserts near a gene that is important in a critical cellular process like growth or division, and this insertion results in the development of a cancer (often leukemia). Using molecular diagnostic techniques, it was determined that clones from these subjects showed retrovirus insertion in proximity to the promoter of the LMO2 proto-oncogene. Earlier generation retroviruses like the one used in these two studies have been shown to preferentially integrate in regulatory regions of genes that control cell growth.

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These well-publicized adverse events led to the development of new viral vectors, such as lentiviral vectors, with improved safety profiles. In published studies, lentiviral vectors have demonstrated an improved safety profile over gamma-retroviral vectors, with no known events of gene therapy-related adverse events, which we believe is due to a number of factors including the tendency of these vectors to integrate within genes rather than in areas that control gene expression, as well as their lack of strong viral enhancers. However, it should be noted that in our Phase I/II study (the LG001 Study) of autologous HSCs transduced *ex vivo* using an earlier generation of our LentiGlobin vector, called HPV569, we initially observed in one patient that a disproportionate number of the cells expressing our functional gene had the same insertion site. Tests showed that this partial clonal dominance contained an insertion of the functional gene in the HMGA2 gene that persisted for a period of two to three years. Although there was some initial concern that the observed clonal dominance might represent a pre-leukemic event, there have been no adverse clinical consequences of this event, or any signs of cancer, in over five years since the observation was made. The presence of the HMGA2 clone has steadily declined in this patient over time to the point that it is no longer the most common clone observed in this patient.

The risk of insertional oncogenesis remains a significant concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical studies. There is also the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. The FDA has stated that lentiviral vectors possess characteristics that may pose high risks of delayed adverse events. If any such adverse events occur, further advancement of our clinical studies could be halted or delayed, which would have a material adverse effect on our business and operations.

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

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory advisory group or authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. For example, the development of our product candidates for pediatric use is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

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

Even if we obtain regulatory approval in a jurisdiction, the regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the FDA typically advises that patients treated with gene therapy undergo follow-up observations for potential adverse events for a 15-year period. Additionally, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

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

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

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

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

**Risks related to our reliance on third parties**

***We expect to rely on third parties to conduct some or all aspects of our vector production, product manufacturing, protocol development, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.***

We do not expect to independently conduct all aspects of our vector production, product manufacturing, protocol development, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these items.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical studies are conducted in accordance with the study plan and protocols.

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

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to clinical study delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

***We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.***

We currently have relationships with a limited number of suppliers for the manufacturing of our viral vectors and product candidates. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical studies must be manufactured in accordance with GMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's good laboratory practices, or GLP, and GMP regulations enforced by the FDA through its facilities inspection program. Some of our contract manufacturers have not produced a commercially-approved product and therefore have not obtained the requisite FDA approvals to do so. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

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

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

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

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

***We expect to rely on third parties to conduct, supervise and monitor our clinical studies, and if these third parties perform in an unsatisfactory manner, it may harm our business.***

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

We and our CROs are required to comply with the FDA's GCPs for conducting, recording and reporting the results of clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical study participants are protected. The FDA enforces these GCPs through periodic inspections of study sponsors, principal investigators and clinical study sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our future clinical studies may be deemed unreliable and the FDA may require us to perform additional clinical studies before approving any marketing applications. Upon inspection, the FDA may determine that our clinical studies did not comply with GCPs. In addition, our future clinical studies will require a sufficient number of test subjects to evaluate the safety and efficacy of our product candidates. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such clinical studies, which would delay the regulatory approval process.

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

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

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

Because we rely on third parties to manufacture our vectors and our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our gene therapy platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These

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agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

### **Risks related to commercialization of our product candidates**

***We intend to rely on third-party manufacturers to produce our vector, product candidates and other key materials, but we have not entered into binding agreements with any such manufacturers to support commercialization. Additionally, these manufacturers do not have experience producing our vectors and product candidates at commercial levels and may not achieve the necessary regulatory approvals or produce our vectors and products at the quality, quantities, locations and timing needed to support commercialization.***

We have not yet secured manufacturing capabilities for commercial quantities of our viral vectors or established transduction facilities in the desired commercialization regions to support commercialization of our products. Although we intend to rely on third-party manufacturers for commercialization, we have only entered into agreements with such manufacturers to support our clinical studies. We may be unable to negotiate binding agreements with the manufacturers to support our commercialization activities at commercially reasonable terms.

No manufacturer currently has the experience or ability to produce our vectors and product candidates at commercial levels. We are currently developing a scalable manufacturing process for LentiGlobin, which we plan to transfer to one or more contract manufacturers. We may run into technical or scientific issues related to manufacturing or development that we may be unable to resolve in a timely manner or with available funds. Although we have been able to produce our Lenti-D vector at commercial scale, we have not completed the characterization and validation activities necessary for commercial and regulatory approvals. If our manufacturing partners do not obtain such regulatory approvals, our commercialization efforts will be harmed.

Additionally, since the HSCs have a limited window of stability following extraction from the patient, we must set up transduction facilities in the regions where we wish to commercialize our product. Currently, we rely on academic institutions and one third-party contract manufacturer in the United States and Europe, respectively, to produce our product candidates for our clinical studies. Since a portion of our target patient populations will be outside the United States and Europe, we will need to set up additional transduction facilities that can replicate our transduction process. Establishment of such facilities may be impeded by technical, quality, or regulatory issues related to these new sites and we may also run into technical or scientific issues related to transfer of our transduction process or other developmental issues that we may be unable to resolve in a timely manner or with available funds.

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

In addition, any significant disruption in our supplier relationships could harm our business. We source key materials from third parties, either directly through agreements with suppliers or indirectly through our manufacturers who have agreements with suppliers. There are a small number of suppliers for certain key materials that are used to manufacture our product candidates. Such suppliers may not sell these key materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have any control over the process or timing of the acquisition of these key materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these key materials.



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

We have no experience selling and marketing our product candidates. 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 may enter into collaborations with other entities to utilize their mature marketing and distribution capabilities, but we may be unable to enter into marketing agreements on favorable terms, if at all. If our future collaborative partners do not commit sufficient resources to commercialize our future products, if any, and we are unable to develop the necessary marketing capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

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

We are engaged in gene therapy, which is a rapidly changing field. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Some of the pharmaceutical and biotechnology companies we expect to compete with include GlaxoSmithKline plc, Sangamo BioSciences Inc., HemaQuest Pharmaceuticals, Inc., Merck & Co., Inc., Novartis AG and GlycoMimetics Inc. In addition, many universities and private and public research institutes are active in our target disease areas.

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

Even if we are successful in achieving regulatory approval to commercialize a product candidate faster than our competitors, we may face competition from biosimilars due to the changing regulatory environment. In the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biological products that are demonstrated to be “highly similar,” or biosimilar, to or “interchangeable” with an FDA-approved biological product. This new pathway could allow competitors to reference data from biological products already approved after 12 years from the time of approval. In his proposed budget for fiscal year 2014, President Obama proposed to cut this 12-year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity due to minor changes in product formulations, a practice often referred to as “evergreening.” In Europe, the European Commission has granted marketing authorizations for several biosimilars pursuant to a set of general and product class-specific guidelines for biosimilar approvals issued over the past few years. In Europe, a competitor may reference data from biological products already approved, but will not be able to get on the market until 10 years after the time of approval. This 10-year period will be extended to 11 years if, during the first eight of those 10 years, the marketing authorization holder obtains an approval for one or more new therapeutic indications that bring significant clinical benefits compared with existing therapies. In addition, companies may be developing biosimilars in other countries that could compete with our products. If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences. Expiration or successful challenge of our applicable patent rights could also trigger competition from other products, assuming any relevant exclusivity period has expired.

In addition, although our product candidates have been granted orphan drug status by the FDA and EMA, there are limitations to the exclusivity. In the United States, the exclusivity period for orphan drugs is seven years, while pediatric exclusivity adds six months to any existing patents or exclusivity periods. In Europe, orphan drugs may be able to obtain 10 years of marketing exclusivity and up to an additional two years on the basis of qualifying pediatric studies. However, orphan exclusivity may be reduced to six years if the drug no longer satisfies the original designation criteria. Additionally, a marketing authorization holder may lose its orphan exclusivity if it consents to a second orphan drug application or cannot supply enough drug. Orphan drug exclusivity also can be lost when a second applicant demonstrates its drug is “clinically superior” to the original orphan drug.

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

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***The commercial success of any current or future product candidate will depend upon the degree of market acceptance by physicians, patients, third-party payors and others in the medical community.***

Ethical, social and legal concerns about gene therapy and genetic research could result in additional regulations restricting or prohibiting the products and processes we may use. Even with the requisite approvals, the commercial success of our product candidates will depend in part on the medical community, patients, and third-party payors accepting gene therapy products in general, and our product candidates in particular, as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of these product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the potential efficacy and potential advantages over alternative treatments;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the prevalence and severity of any side effects resulting from the chemotherapy and myeloablative treatments associated with the procedure by which our product candidates are administered;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage or reimbursement.

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

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

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

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

***The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.***

The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford expensive treatments, such as stem cell transplants. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.



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There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. It is difficult to predict what CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries. In addition, costs or difficulties associated with the reimbursement of Glybera could create an adverse environment for reimbursement of other gene therapies.

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

Moreover, increasing efforts by governmental and third-party payors, in the United States and abroad, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

***Due to the novel nature of our technology and the potential for our product candidates to offer therapeutic benefit in a single administration, we face uncertainty related to pricing and reimbursement for these product candidates.***

Our target patient populations are relatively small, as a result of which the pricing and reimbursement of our product candidates, if approved, must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our products.

***If the market opportunities for our product candidates are smaller than we believe they are, our revenues may be adversely affected and our business may suffer. Because the target patient populations of our product candidates are small, we must be able to successfully identify patients and achieve a significant market share to maintain profitability and growth.***

We focus our research and product development on treatments for severe genetic and orphan diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. The number of patients in the United States, Europe and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

## **Risks related to our business operations**

***Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.***

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, in 2003, 20 subjects treated for X-linked severe combined immunodeficiency in two gene therapy studies using a murine gamma-retroviral vector showed correction of the disease, but the studies were terminated after five subjects developed leukemia (four of whom were

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subsequently cured). Although none of our current product candidates utilize these gamma-retroviruses, our product candidates use a viral delivery system. Adverse events in our clinical studies, even if not ultimately attributable to our product candidates (such as the many adverse events that typically arise from the transplant process) and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

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

We are highly dependent on principal members of our executive team and key employees, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are “at will” employees. Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled executives in our industry, which is likely to continue. As a result, competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical studies may make it more challenging to recruit and retain qualified personnel. The inability to recruit or loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives.

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

As of September 30, 2013, we had 76 full-time employees. As we mature and undertake the activities required under our collaboration with Celgene, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. For example, in the past there have been errors in the preparation of our financial statements and there can be no assurance that other errors will not occur in the future as we grow. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

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

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

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

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

- impairment of our business reputation;
- withdrawal of clinical study participants;
- costs due to related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates; and
- decreased demand for our product candidates, if approved for commercial sale.

We carry product liability insurance of \$10 million per occurrence and \$10 million aggregate limit. We believe our product liability insurance coverage is sufficient in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

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

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

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

The success of our business depends primarily upon our ability to identify, develop and commercialize products based on our gene therapy platform. Although our Lenti-D and LentiGlobin product candidates are currently in clinical development, our research programs, including those subject to our collaboration with Celgene, may fail to identify other potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

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

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

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

***We incur significant increased costs as a result of operating as a public company, and our management devotes substantial time to new compliance initiatives.***

As a public company, we have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, as well as rules subsequently implemented by the Securities and Exchange Commission, or SEC, and The Nasdaq Global Market have imposed various requirements on public companies. In July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Recent legislation permits us, as a smaller “emerging growth company,” to implement many of these requirements over a longer period and up to five years from the pricing date of our initial public offering, which was June 18, 2013. We are taking advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such coverage.

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

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

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

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of

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our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business.

### **Risks related to our intellectual property**

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

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

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

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

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

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Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

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

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

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

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

Presently we have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our gene therapy product candidates. Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

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

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



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

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

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. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. 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;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- 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.

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

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

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

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

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

On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The U.S. PTO is currently developing regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, were enacted March 16, 2013. However, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

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

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

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

***Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.***

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the



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validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

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

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore obtaining and enforcing biotechnology patents is costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the U.S. PTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

***We have not yet registered trademarks for a commercial trade name for Lenti-D and failure to secure such registrations could adversely affect our business.***

We have not yet registered trademarks for a commercial trade name for Lenti-D. During trademark registration proceedings, we may receive rejections. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the U.S. PTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we propose to use with our product candidates in the United States must be approved by the FDA and the EMA in the European Union, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA and EMA typically conduct a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA or EMA object to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA and EMA.

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

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

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

**Risks related to ownership of our common stock**

***The market price of our common stock may be highly volatile, and you may not be able to resell your shares at or above the price at which you purchase them.***

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

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

- adverse results or delays in preclinical or clinical studies;
- reports of adverse events in other gene therapy products or clinical studies of such products;
- inability to obtain additional funding;
- any delay in filing an IND or BLA for any of our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND or BLA;
- failure to develop successfully and commercialize our product candidates;
- failure to maintain our existing strategic collaborations or enter into new collaborations;
- failure by us or our licensors and strategic collaboration partners to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to future products;
- inability to obtain adequate product supply for our product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- introduction of new products, services or technologies by our competitors;
- failure to meet or exceed financial projections we may provide to the public;
- failure to meet or exceed the financial projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us, our strategic collaboration partner or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;
- changes in the market valuations of similar companies;
- sales of our common stock by us or our stockholders in the future; and
- trading volume of our common stock.

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

Our executive officers, directors, five percent stockholders and their affiliates beneficially own approximately 48.8% of our outstanding voting stock. Therefore, these stockholders will have the ability to influence us through their ownership positions. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

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

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to approximately five years (December 31, 2018), although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds \$700.0 million as of any June 30 before that time or if we have total annual gross revenue of \$1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than \$1.0 billion in non-convertible debt during any three-year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a

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“smaller reporting company” which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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

***Our stock price could decline due to the large number of outstanding shares of our common stock eligible for future sale.***

Sales of a substantial number of shares of our common stock in the public market or the market perception that the holder or holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. These sales could also make it more difficult for us to sell equity or equity-related securities in the future at a time and price that we deem appropriate.

As of September 30, 2013, we had an aggregate of 23,722,009 (which includes 89,496 shares of unvested restricted stock subject to repurchase by us) shares of common stock outstanding, of which approximately 6.9 million are freely tradeable, without restriction in the public market. An additional 16.8 million shares will be eligible for sale upon the expiration of lock-up agreements with us or with the underwriters for our initial public offering, subject in some cases to volume and other restrictions of Rule 144 and Rule 701 under the Securities Act. The lock-up agreements expire on December 15, 2013. J.P. Morgan Securities LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated, as representatives of the underwriters, may, in their discretion and at any time, release all or any portion of the securities subject to lock-up agreements with the underwriters. We have registered approximately 5.1 million shares of our common stock that have been issued or reserved for future issuance under our stock incentive plans and may register up to 16.3 million shares of our common stock held by holders of registration rights. Once we register the offer and sale of shares for the holders of registration rights and option holders, they can be freely sold in the public market upon issuance, subject to the lock-up agreements, unless they are held by “affiliates,” as that term is defined in Rule 144 of the Securities Act.

We may also issue shares of our common stock or securities convertible into our common stock from time to time in connection with a financing, acquisition or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and cause the trading price of our common stock to decline.

***Actual or potential sales of our common stock by our employees, including our executive officers, pursuant to pre-arranged stock trading plans could cause our stock price to fall or prevent it from increasing for numerous reasons, and actual or potential sales by such persons could be viewed negatively by other investors.***

In accordance with the guidelines specified under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended, and our policies regarding stock transactions, a number of our employees, including executive officers, have adopted and may continue to adopt stock trading plans pursuant to which they have arranged to sell shares of our common stock from time to time in the future. Generally, sales under such plans by our executive officers and directors require public filings. Actual or potential sales of our common stock by such persons could cause the price of our common stock to fall or prevent it from increasing for numerous reasons. For example, a substantial number of shares of our common stock becoming available (or being perceived to become available) for sale in the public market could cause the market price of our common stock to fall or prevent it from increasing. Also, actual or potential sales by such persons could be viewed negatively by other investors.

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

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

Pursuant to our 2013 Stock Option and Incentive Plan, or the 2013 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares available for future grant under the 2013 Plan will automatically increase each year by up to 4% of all shares of our capital stock outstanding as of December 31 of the prior calendar year, subject to the ability of our board of directors or compensation committee to take action to reduce the size of the increase in any given year. Currently, we plan to register the increased number of shares available for issuance under the 2013 Plan each year. If our board of directors or compensation committee elects to increase the number of shares available for future grant by the maximum amount each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

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***We could be subject to securities class action litigation.***

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

***We have broad discretion in the use of the net proceeds we received from our initial public offering and may not use them effectively.***

Our management has broad discretion in the application of the net proceeds from our initial public offering, and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from our initial public offering, their ultimate use may vary substantially from their currently intended use. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from our initial public offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders.

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

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We believe we may have triggered an "ownership change" limitation. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

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

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

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

Our amended and restated certificate of incorporation, amended and restated by-laws and Delaware law contain provisions that may have the effect of delaying or preventing a change in control of us or changes in our management. Our amended and restated certificate of incorporation and by-laws, include provisions that:

- authorize "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors, the chairperson of our board of directors, our chief executive officer or our president;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that our directors may be removed only for cause;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated by-laws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated by-laws.

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These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us.

Any provision of our amended and restated certificate of incorporation or amended and restated by-laws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

***Provisions in our collaboration agreement with Celgene Corporation may prevent or delay a change in control.***

Our collaboration agreement with Celgene Corporation provides that during the initial three-year term of the collaboration and, if extended, during the first extension term of the collaboration which is two years, in the event that we engage in a change in control transaction, including for such purposes a merger or consolidation of bluebird bio or the sale of all or substantially all of our assets, or if another person or entity or group of persons or entities acquires at least 50% of our voting capital stock, then Celgene has the right, but not the obligation, to terminate the collaboration agreement and obtain perpetual, non-terminable, worldwide, exclusive, fully paid-up licenses to all, but not less than all, of the product candidates previously identified under the collaboration agreement. We refer to this right to acquire such licenses as the call option.

Under the call option, the product candidates to which Celgene would have the right to acquire fully paid-up licenses include any product candidate previously licensed out of the collaboration during the term of the collaboration, any product candidate for which we have exercised our right to co-develop and co-promote the product candidate within the United States, any product candidate for which Celgene previously declined its option to obtain a license and any product candidate for which at least *in vivo* efficacy studies have been initiated or authorized by the joint steering committee for the collaboration. The purchase price for such fully paid-up licenses would be determined pursuant to a binding arbitration process and would be paid on or about the consummation of the change of control transaction with our acquiror. The call option will lapse at the end of the three-year term of the collaboration, unless extended, in which case it will lapse at the end of the first extension term, which is two years, even if the collaboration is extended further.

In addition, during the initial three-year term of the collaboration, but not during any extension of the collaboration agreement, in the event that we engage in a change in control transaction described above and Celgene exercises the call option described above, then, in addition to the right to acquire the fully paid-up licenses described above, Celgene would also have the right to obtain a perpetual, non-terminable, worldwide, exclusive license to our intellectual property to develop one or more CAR T cell products targeting one or more oncology associated target antigens identified by Celgene following the third anniversary of the collaboration agreement. There is no limit to the number of oncology associated target antigens Celgene may select under this license. Upon commercialization of any such product candidate so licensed by Celgene, Celgene would be obligated to pay us a specified milestone payment upon regulatory approval and a percentage of net sales as a royalty. We refer to this license agreement to develop one or more CAR T cell products targeting one or more oncology associated target antigens as the target antigen license. The right to acquire a target antigen license will lapse after the initial three-year term of the collaboration, even if the collaboration is extended.

The call option and the right to acquire a target antigen license may have the effect of delaying or preventing a change in control transaction involving us, or may reduce the number of companies interested in acquiring us. If Celgene were to exercise the call option, it would gain exclusive development and marketing rights to the product candidates developed under the collaboration agreement, including any product for which we previously exercised our co-development and co-promotion rights. Were this to happen, our successor would not receive a royalty on net sales of any of the products out-licensed in connection with the call option, nor would it realize any value it may otherwise ascribe to our right to co-develop and co-promote within the United States any products developed during the collaboration. Moreover, if such event were to occur during the first three years of the collaboration, Celgene would also effectively have the exclusive right to develop and market an unlimited number of additional CAR T cell products using our gene therapy platform, whether or not these products were first identified or developed during the course of the collaboration, which product candidates would target a list of oncology associated target antigens that would not be known at the time we close our change in control transaction. This license could potentially give Celgene rights to our gene therapy platform for CAR T cell product candidates in the event we are acquired prior to the third anniversary of the collaboration.

These provisions could have the effect of delaying or preventing a change in control transaction involving bluebird bio, or could reduce the number of companies interested in acquiring us, in particular during the first three years of the collaboration. This risk may become particularly acute in the event either of our lead product candidates, Lenti-D or LentiGlobin, suffer material setbacks or delays in their clinical advancement, as a result of which the long-term strategic value potential acquirors may ascribe to us could increasingly be attributable to the potential long-term value of any CAR T cell products we develop under the collaboration.

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**Item 2. Unregistered Sales of Equity Securities and Use of Proceeds**

***Use of Proceeds from Initial Public Offering of Common Stock***

On June 24, 2013, we closed the sale of 6,832,352 shares of common stock to the public (inclusive of 891,176 shares of common stock sold by us pursuant to the full exercise of an overallotment option granted to the underwriters) at a price of \$17.00 per share. The offer and sale of the shares in the IPO was registered under the Securities Act pursuant to registration statements on Form S-1 (File No. 333-188605), which was filed with the Securities and Exchange Commission, or the SEC, on May 14, 2013 and amended subsequently and declared effective on June 18, 2013, and Form S-1MEF (File No. 333-189430), which was filed with the SEC on June 18, 2013 and declared effective on June 18, 2013. J.P. Morgan Securities LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated acted as managing underwriters of the offering.

We raised approximately \$104.9 million in net proceeds after deducting underwriting discounts and commissions of approximately \$8.1 million and other offering expenses of approximately \$3.1 million. None of these expenses consisted of payments made by us to directors, officers or persons owning 10% or more of our common stock or to their associates, or to our affiliates. There has been no material change in the planned use of proceeds from our IPO as described in our final prospectus filed with the SEC on June 19, 2013 pursuant to Rule 424. We invested the funds received in cash equivalents and other short-term investments in accordance with our investment policy. As of September 30, 2013, the entire amount of the net proceeds is included as cash and cash equivalents.

**Item 5. Other Information**

Our policy governing transactions in our securities by our directors, officers, and employees permits our officers, directors and certain other persons to enter into trading plans complying with Rule 10b5-1 under the Securities Exchange Act of 1934, as amended. We have been advised that certain of our officers (including Nick Leschly, President and Chief Executive Officer, Jeffrey Walsh, Chief Operating Officer, Mitchell Finer, Chief Scientific Officer, David Davidson, Chief Medical Officer, and Linda Bain, Vice President, Finance and Business Operations) have entered into trading plans covering periods after the date of this quarterly report on Form 10-Q in accordance with Rule 10b5-1 and our policy governing transactions in our securities. Generally, under these trading plans, the individual relinquishes control over the transactions once the trading plan is put into place. Accordingly, sales under these plans may occur at any time, including possibly before, simultaneously with, or immediately after significant events involving our company. We do not undertake to report Rule 10b5-1 trading plans that may be adopted by any officers or directors in the future, or to report any modifications or termination of any publicly announced trading plan, except to the extent required by law.

**Item 6. Exhibits**

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

**SIGNATURES**

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

bluebird bio, Inc.

Date: November 14, 2013

By: /s/ Nick Leschly

Nick Leschly  
*President, Chief Executive Officer and Director (Principal Executive Officer and Duly Authorized Officer)*

Date: November 14, 2013

By: /s/ Jeffrey T. Walsh

Jeffrey T. Walsh  
*Chief Operating Officer and Secretary (Principal Financial Officer and Duly Authorized Officer)*



**Exhibit Index**

| <b>Exhibit Number</b> | <b>Description of Exhibit</b>  |
|-----------------------|--|
| 3.1                   | Amended and Restated Certificate of Incorporation of bluebird bio, Inc., effective as of June 24, 2013 (incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed on June 24, 2013).  |
| 3.2                   | Amended and Restated By-Laws of bluebird bio, Inc., effective as of June 24, 2013 (incorporated by reference to Exhibit 3.2 to the Company's Form 8-K filed on June 24, 2013).   |
| 4.1                   | Form of Common Stock Certificate of bluebird bio, Inc. (incorporated by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1 (File No. 333-188605) filed on June 4, 2013).   |
| 10.1*                 | Amended and Restated Lease Agreement, dated May 18, 2007, by and between the Company and Rivertech Associates II, LLC, as amended.   |
| 10.2*†                | Amendment No. 3 to License Agreement, dated September 10, 2013, by and between the Company and Institut Pasteur  |
| 31.1*                 | Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.  |
| 31.2*                 | Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.  |
| 32.1**                | Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.   |
| 101***                | The following materials from the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2013, formatted in XBRL (eXtensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets as of September 30, 2013 and December 31, 2012, (ii) Condensed Consolidated Statements of Operations and Comprehensive Loss for the three and nine months ended September 30, 2013 and 2012, (iii) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2013 and 2012 and (iv) Notes to Unaudited Condensed Consolidated Financial Statements. |

\* Filed herewith.

\*\* Furnished herewith.

\*\*\* As provided in Rule 406T of Regulation S-T, this information is furnished and not filed for purposes of Sections 11 and 12 of the Securities Act of 1933 and Section 18 of the Securities Exchange Act of 1934.

† Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment and this exhibit has been submitted separately to the SEC.

**RIVERSIDE TECHNOLOGY CENTER  
AMENDED AND RESTATED LEASE AGREEMENT**

Agreement entered into this 18<sup>th</sup> day of May, 2007 by and between **Rivertech Associates II, LLC**, a Massachusetts limited liability company with a principal address at The Abbey Group, 575 Boylston Street Boston, Massachusetts (the "LESSOR"), and **Genetix Pharmaceuticals, Inc.** a corporation with a principal address at 840 Memorial Drive Cambridge, Massachusetts (the "LESSEE"); relative to certain space in the building owned by the LESSOR at 840 Memorial Drive Cambridge, Massachusetts (the "Building"), as follows:

**WHEREAS**, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain lease agreement dated February 18, 2000 (the "Original Lease"); and,

**WHEREAS**, LESSOR and LESSEE each hereby represent to the other that as of the date hereof the foregoing Original Lease represents the full and complete agreement relative to LESSEE'S use and occupancy of certain space described thereunder, consisting (prior to this "Amended and Restated Lease Agreement") of approximately 10,593 rentable square feet of space on the fifth (5<sup>th</sup>) floor of the Building, which space is specifically referred to herein as the "Current Leased Premises"; and,

**WHEREAS**, certain space consisting of approximately 10,500 rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building (the "Substituted Leased Premises"), is available as of June 1, 2007 and LESSOR has reached agreement with LESSEE for LESSEE to surrender the Current Leased Premises as of the date (the "Substitution Date") the Substituted Leased Premises are delivered to Tenant in accordance with Section 5 hereof (anticipated to be June 1, 2007), and to lease the Substituted Leased Premises as of the Substitution Date under the terms and conditions of the Lease as revised and amended by this Amended and Restated Lease Agreement; and,

**NOW THEREFORE**, for One (\$1.00) Dollar and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the parties hereto agree as follows:

**1. Original Term Expiration and Commencement of the Extended Lease Term**

The current Term under the Original Lease, prior to this Amended and Restated Lease Agreement, expires on June 30, 2007. Effective as of the Substitution Date, LESSEE shall vacate and surrender the Current Leased Premises to LESSOR and LESSOR shall accept such surrender from LESSEE and the Original Lease shall be deemed terminated provided that Subtenant shall have access to the Current Leased Premises for a period of up to thirty (30) days after the Substitution Date in order to satisfy the provisions of the Original Lease pertaining to the condition in which the Current Leased Premises are to be delivered to LESSOR (e.g. Lease Sections 26 and 28), and to effectuate the transfer of LESSEE'S equipment and furniture from the Current Leased Premises to the Substituted Leased Premises in a timely and orderly manner.

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As of the Substitution Date, the Term of the Original Lease (as amended by this Amended and Restated Lease Agreement) will be extended for a period beginning on the Substitution Date and expiring on May 31, 2010 (the "Extended Term"); and LESSEE shall lease the Substituted Leased Premises under the Rent, terms and conditions as set forth in this Amended and Restated Lease Agreement up to the expiration of said Extended Term.

To the extent LESSOR cannot deliver the Substituted Leased Premises to LESSEE on or prior to June 30, 2007, the term of the Original Lease shall be automatically extended through that date which subsequently becomes the Substitution Date, except that the Rent thereunder shall be amended to be equal to the Rent for the Substituted Leased Premises during the first Lease Year of the Extended Term.

The term "Lease Year" as used herein and for all purposes during the Extended Term shall mean the period beginning on the Substitution Date and ending on the day immediately prior to each anniversary of the Substitution Date, provided that the Third Lease Year shall end on May 31, 2010.

## **2. The Substituted Leased Premises**

LESSOR and LESSEE hereby agree that during the Extended Term, the Substituted Leased Premises, as defined herein and as depicted on Exhibit A attached hereto, shall be deemed to mean the "Leased Premises" as referred to in the Lease, unless the context specifically means the "Current Leased Premises."

The Substituted Leased Premises may be used by LESSEE for general office, research and laboratory uses only, as set forth in Section 6 of the Lease, throughout the Extended Term.

Provided the same does not materially interfere with LESSOR'S obligations under Section 5 hereof (in which case LESSEE'S rights under this paragraph shall be expressly subordinate in LESSOR'S sole discretion). LESSEE shall have reasonable supervised access to the Substituted Leased Premises commencing on the date of this Agreement to install trade fixtures and/or cabling and/or communications equipment, and to reconfigure and move laboratory and office equipment. Such early access by LESSEE shall be subject to such times and in such manner as LESSOR may reasonably determine to avoid material interference with LESSOR'S Work.

LESSEE shall not be charged any fees by LESSOR for use of the freight elevators of the building, supervision or otherwise in connection with LESSEE'S move from the Current Leased Premises to the Substituted Leased Premises.

LESSOR shall provide the services of LESSOR'S internal space planning and design staff, at LESSOR'S cost and expense, on a reasonable basis to assist LESSEE in its own space planning and design.

## **3. Annual Base Rent—Revised Annual Base Rent Schedule**

Commencing as of the beginning of the Extended Term, LESSEE'S monthly installments of Annual Base Rent for the Leased Premises shall be based on Annual Base Rent as set forth in the "Revised Annual Base Rent Schedule" appearing below.

**Revised Annual Base Rent Schedule**

|                   |                                  |
|-------------------|----------------------------------|
| First Lease Year  | \$ 388,500.00 (\$ 32,375.00/mo.) |
| Second Lease Year | \$ 399,000.00 (\$ 33,250.00/mo.) |
| Third Lease Year  | \$ 420,000.00 (\$ 35,000.00/mo.) |

Annual Base Rent shall be payable by Lessee for each Lease Year of the Extended Term as set forth above in twelve installments during each Lease Year (reflecting the aforesaid Revised Annual Base Rent Schedule), in advance, on or before the first day of each calendar month.

Except as otherwise set forth in Section I on account of the Substitution Date occurring after June 30, 2007, LESSEE shall continue to pay Annual Base Rent under the Original Lease for the Current Leased Premises up to the Substitution Date.

**4. Additional Rent**

Notwithstanding the provisions of the Original Lease, this Amended and Restated Lease Agreement is structured as a “triple net” lease. Accordingly, the provisions for Additional Rent based on Operating Expenses and Taxes are rewritten as follows.

LESSEE shall also pay to LESSOR, as Additional Rent under the Lease, the following amounts based on LESSEE’S allocable percentage (which is 8.3441%, the “Allocable Percentage”):

A. Operating Expenses: LESSEE shall be responsible for payment of Additional Rent attributable to the Operating Expenses for the Building and site, based on LESSEE’S Allocable Percentage. Operating Expenses, as set forth in Exhibit B hereto, are the unaudited actuals for calendar year 2006 (and will be subject to change based on actual costs and expenses incurred for each of the categorized Exhibit B costs and expenses in 2007 and each subsequent year during the Extended Term). “Operating Expenses” shall not include the following: the costs of LESSEE’S improvements and services for which LESSEE or any tenant specifically and directly reimburses LESSOR, or pays third persons at LESSOR’S directions; income or franchise taxes of the LESSOR; the costs incurred in any rehabilitation, reconstruction or other work occasioned by any insured casualty (i.e. as to which LESSOR is required to carry insurance hereunder), or by the exercise of the right of eminent domain (except to the extent of any so-called “deductible” amount under policies of insurance or any costs actually incurred for which any insurance company does not reimburse or compensate LESSOR or Owner); depreciation of the Building; general corporate overhead of the LESSOR entity; legal expenses incurred in any direct dispute with any particular tenant (other than those incurred which are of benefit to or protect the rights of other tenants in the Building, generally); costs of renovations to other tenants’ spaces; costs of capital improvements; brokerage and advertising costs in seeking new tenants; and penalties incurred due to LESSOR’S willful violation of any direct violation of any government order.

B. Tax Expenses: LESSEE shall be responsible for payment of Additional Rent attributable to the municipal real estate taxes on the Building and land on which it is situated, based on LESSEE’S Allocable Percentage.

Additional Rent for Operating Expenses and Tax Expenses shall be payable as invoiced by LESSOR (accompanied by a copy of the applicable municipal tax bill), and failure to make any such payments within thirty (30) days of such invoice shall be a default under this Amended and Restated Lease Agreement.

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C. Utilities: Landlord shall provide and Tenant shall pay utilities attributable to the Substituted Leased Premises as set forth in Section 7 of the Original Lease.

**5. Condition of the Current Leased Premises on the Substitution Date; and Landlord's Work on the Substituted Leased Premises**

The Current Leased Premises shall be vacated and surrendered by the LESSEE in accordance with the provisions of Sections 26 and 28 of the Lease, upon delivery of the Substituted Leased Premises to the LESSEE by the LESSOR as contemplated herein.

LESSOR and LESSEE acknowledge that LESSOR shall deliver and LESSEE shall accept delivery of the Substituted Leased Premises as of the beginning of the Extended Term in an AS/IS condition in all respects, but for: (a) the specific work to be performed by LESSOR as set forth in Exhibit C hereto (the "LESSOR'S Work"); (b) in vacant and clean condition; (c) with the laboratory areas "decommissioned" (i.e. free of any environmental hazards or materials such that it is not in violation of applicable environmental laws consistent with the same standards set forth in Section 26 of the Lease) and sanitized. Landlord represents that the Substituted Leased Premises currently has the benefit of a certificate of occupancy from the City of Cambridge therefor which permits the use of the Substituted Leased Premises for the uses permitted under this Lease, and to the extent any additional building permit (or resulting certificate of occupancy is required on account of LESSOR'S Work), then LESSOR shall be required to procure and deliver the same to LESSEE. LESSOR'S Work shall be performed at LESSOR'S sole cost and expense, and shall be substantially completed as of the beginning of the Extended Term. LESSOR shall perform LESSOR'S Work in a good and workmanlike manner and in compliance with all applicable laws and governmental regulations. LESSEE shall inspect such work upon delivery by LESSOR, and hereby acknowledges there are no special installations or other requirements with respect thereto which do not appear on Exhibit C hereto. Notwithstanding LESSOR'S obligation to perform LESSOR'S Work, LESSOR shall not be liable for any damages resulting from LESSEE'S transfer of its operations and equipment from the Current Leased Premises to the Substituted Leased Premises or performance of any of its business functions (e.g. laboratory work) from the newly installed facilities in the Substituted Leased Premises; LESSEE assuming all such risk and waiving any and all claims against LESSOR with respect thereto. If LESSOR is unable to fulfill the conditions set forth above within one hundred twenty (120) days after June 15, 2007, then LESSEE, as its sole and exclusive remedy at law or in equity shall be entitled to terminate this Amended and Restated Lease Agreement by written notice to LESSOR delivered within ten (10) days of the expiration of said one hundred twenty (120) day period, and this Amended and Restated Lease Agreement shall be null and void and without recourse to either party, but LESSEE shall be entitled to remain in occupancy of the Current Leased Premises at the rent set forth in this Amended and Restated Lease Agreement for ninety (90) days after such termination.

LESSOR shall be solely responsible for any costs associated with the architectural and engineering work and permits required for LESSOR'S Work; and LESSOR shall provide such architectural and engineering and permitting services as part of LESSOR'S Work.

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**6. Security/Guaranty**

The LESSEE'S obligations to post and maintain a Security Deposit under Section 5 of the original Lease shall also be required during the Extended Term.

**7. Parking**

The provisions of Section 16 of the Lease shall continue to govern LESSEE'S parking rights and LESSEE shall be entitled to the same number of spaces set forth therein. LESSEE acknowledges that the current rate for such parking spaces is \$ 210.00 per space per month. However, notwithstanding the provisions of Section 16 of the Lease, LESSOR shall determine from time to time in its discretion the extent to which spaces are provided in the LESSOR'S Building or at 808 Memorial Drive (provided all the spaces contemplated in said Section 16 are provided from one location or the other, with a minimum of fifteen (15) to be provided at 840 Memorial Drive).

**8. Brokers Commissions/Indemnification**

LESSOR has retained Meredith & Grew, and LESSEE has had some dealings with said brokerage firm, relative to the Building. The LESSOR and LESSEE each represent to the other that they have not dealt, directly or indirectly, with any other broker, or other entity or individual entitled to any commission relative to the Substituted Leased Premises leased to LESSEE for the Extended Term hereunder. Each party agrees to indemnify and hold harmless the other from and against any claims for commission arising out the execution and delivery of this agreement and any renewals, extensions or expansions hereof other than the foregoing; LESSOR expressly agreeing that it shall be responsible for any claims made by Meredith & Grew relative to this transaction, based on separate agreement between LESSOR and Meredith & Grew.

**9. Assignment/Subletting**

The LESSEE shall have the right to assign the Lease and/or sublet the Leased Premises (in whole or in part) subject to all the terms and conditions of Section 12 of the Original Lease.

**10. Integration of Documents; Supremacy**

The parties hereto intend that this Amended and Restated Lease Agreement incorporate the provisions of the original Lease (to the extent not specifically superseded by the terms and conditions hereof) and that conjunctively these documents constitute the full and complete agreement as between the parties.

The following provisions of the Original Lease are not applicable to the Extended Term: Section 1 (Term); Section 2 (to the extent of the Schedule of Annual Base Rent); Section 3 (as to the first through fourth paragraphs, only); Section 4 (as to the first, and second, and fourth paragraphs, only); Section 22 (solely to the extent that the notice addresses appearing on the execution pages hereof supersede); Section 33 (which is wholly superseded by Section 5 hereof); and Exhibit B (which is wholly superseded by Exhibit B as it is attached hereto).

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As a condition to the effectiveness of this Agreement, LESSOR shall provide to LESSEE either (a) the written acknowledgement of the current mortgagee that the existing Subordination, Non-Disturbance and Attornment Agreement non-disturbance agreement remains in effect and applies to this Amended and Restated Lease Agreement or (b) a new Subordination, Non-Disturbance and Attornment Agreement in the form attached hereto in favor of LESSEE executed by the current mortgagee with respect to this Amended and Restated Lease Agreement.

This Agreement shall be governed by the laws of the Commonwealth of Massachusetts. Any provisions deemed unenforceable shall be severable, and the remainder of this Agreement shall be enforceable in accordance with its terms. This Agreement may only be modified in writing, signed by both parties. Unless otherwise provided herein, all capitalized terms used herein shall have the same meaning as set forth in the Original Lease.

Witness our hands and seals the first date above written.

**RIVERTECH ASSOCIATES II, LLC**

**By Rivertech Associates, Inc. its duly authorized Manager**

/s/ Robert Epstein

**Robert Epstein, President**

Notice Address: 575 Boylston Street 8<sup>th</sup> Floor  
Boston, Massachusetts 02116  
with a copy to: Christopher C. Tsouros, Esq.  
Posternak Blankstein & Lund LLP  
Prudential Tower  
800 Boylston Street Boston,  
Massachusetts 02199

**GENETIX PHARMACEUTICALS, INC.**

By: /s/ Alfred E. Slanetz

its duly authorized

(Attached Secretary/Clerk's Certificate As To Authority)

Notice Address: 800 Memorial Drive 3<sup>rd</sup> Floor  
Cambridge, Mass.  
with a copy to:



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**GENETIX PHARMACEUTICALS, INC.  
AMENDED AND RESTATED LEASE AGREEMENT**

**REVISED EXHIBIT B**

**Operating Expenses 2006**

840 Memorial Drive—Riverside Technology Center

| <u>DESCRIPTION</u>                 | <u>PSF</u>    |
|------------------------------------|---------------|
| HEAT                               | \$ 0.54       |
| BUILDING ELECTRIC                  | \$2.01        |
| WATER & SEWER                      | \$0.21        |
| ELEVATOR MAINTENANCE               | \$0.15        |
| PARKING/CAFE EXPENSE               | \$0.26        |
| RUBBISH REMOVAL                    | \$0.14        |
| INSURANCE                          | \$0.36        |
| GROUNDS CARE                       | \$0.27        |
| LEGAL/ACCT/ADMIN                   | \$0.11        |
| JANITORIAL SERVICES                | \$0.50        |
| GENERAL MAINTENANCE                | \$1.38        |
| HVAC MAINTENANCE                   | \$0.50        |
| LIFE SAFETY SYSTEMS                | \$0.11        |
| MANAGEMENT                         | \$3.28        |
| <b>Total Operating Expenses</b>    | <b>\$9.82</b> |
| <b>Real Estate Taxes (FY 2007)</b> | <b>\$4.63</b> |

Note: Actual numbers for 2007 and all subsequent years will vary based on actual costs and expenses incurred.

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**THE ABBEY GROUP**

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Genetix Pharmaceuticals  
Relocation to Floor Three, 840 Memorial Drive Revised 5/1/2007

Scope of Work by Landlord

**Office Area**

- All walls to be repainted
- Existing lights to be relamped as required
- Space to be cleaned and prepared for occupancy
- Existing benchtop in support area to be relocated to 3 perimeter offices which presently do not have benchtops.

**Laboratory Area***Physical*

- Repaint all walls
- Vinyl tile floor in main labs and in all non-office areas where seamless tile does not exist.
- New seamless vinyl floor in BL-2A Lab, BL-2B Lab & autoclave/glasswashing room.
- New ceiling and lights throughout lab area.
- Provide existing laboratory cabinets with new bench tops as indicated in the "Floor Plan Exhibit".
- Provide 10 foot long single tier reagent shelving above three benchtops in "Biology Lab A"

*Mechanical*

- Assure proper operation of 10ton supplemental air conditioning supplying main Biology Labs A&B
- Assure proper operation of air conditioning units, humidification and dehumidification system in both BL-2 labs.
- Assure proper operation of cold room.
- Assure proper operation of supplemental air conditioning in equipment room.
- Provide adequate exhaust air for Tenant's (three) six foot hoods, BL2 exhaust hood, BL2 Lab sinks, chemical storage room and autoclave.

*Plumbing*

- Provide separate water supply with approved backflow prevention devices and a hot water tank in or near the location indicated in the "Floor Plan Exhibit".
- Provide drain connection from all sinks to a new acid neutralization system (location to be determined),
- Provide 7 sinks as indicated in plans with eyewash stations. Controls in each of the two BL-2 Labs to be infrared or foot pedal type.
- Provide emergency showers in each BL 2 lab.
- Provide connection of Tenant's glasswasher to cold water, drain -

- 
- Provide connection of Tenant's vacuum and air compressor to existing vacuum and air lines.
  - Provide connection of Tenant's "gas tanks" to Tenant's equipment in the two BL-2 labs.
  - Connect Tenant's ice machine to cold water and indirect drain.

*Electrical*

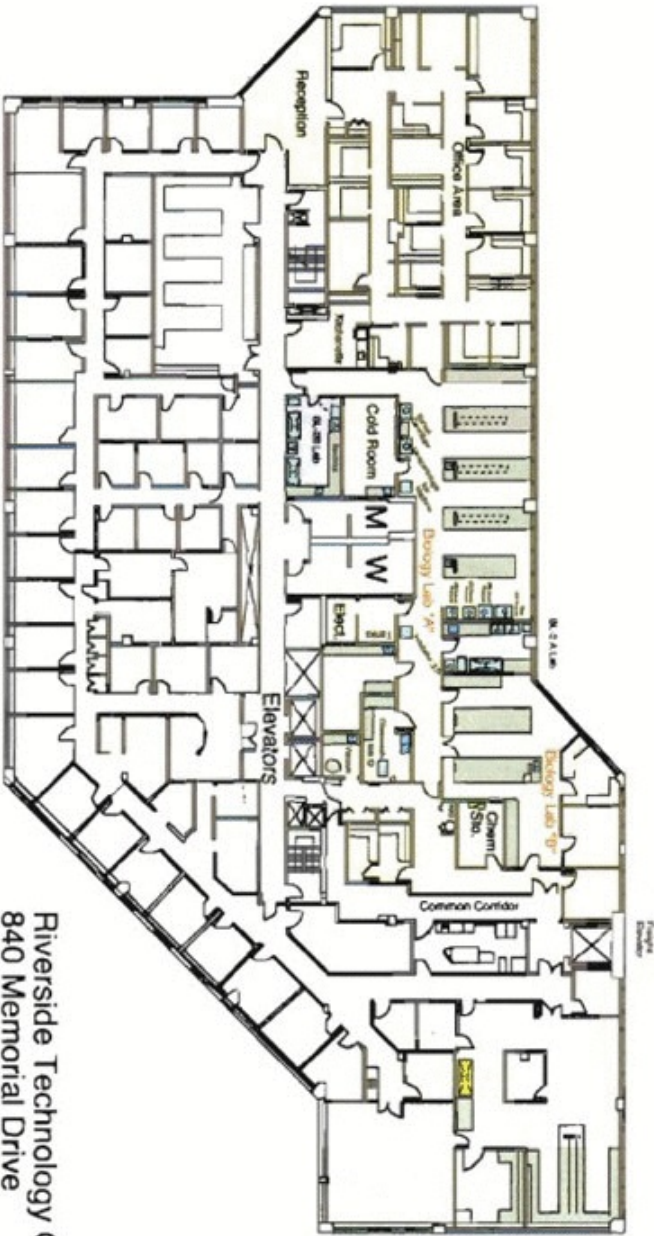
- Provide separate electric service to the premises including submetering of all equipment.
- Provide electric outlets on the three reagent shelving in Lab "A".
- Relocate, Provide power for and install the following Tenant's equipment:
  - Lab Services Area:*
    - glasswasher.
  - Biology Lab "A"*
    - 20 chest freezer, -20 Revco freezer, -20 Bench freezer, incubator 37°, Sorvall centrifuge, ultracentrifuge, shaker & ice machine.
  - BL-2A Lab equipment -*
    - (1) six foot hood, co2 Nuair Incubator, co2 Thermo incubator, refrigerator.
  - BL-2B Lab equipment -*
    - (2) six foot hoods, co2 stack incubator, GS-6K centrifuge, refrigerator.
- NOTE: Emergency power has, in the past, been taken from the building life safety system which is inconsistent with established building policy and must be remedied by the Tenant's installation of a separate emergency generator, most likely on the second floor roof proximate to both the leased premises and a natural gas supply. The Landlord shall assist the tenant by requesting the "building electrician" to provide Tenant a quote for the appropriate work.

*Telecommunications*

- Landlord shall provide consultant to assist Tenant in the relocation of or purchase of new telecommunications system or but Landlord shall not be responsible for cost of either consultation or work associated with IT or telecommunications.

*Relocation of Equipment*

- The landlord shall arrange the move and reconnection of Tenant's large laboratory equipment as specified in equipment list above to the third floor leased premises.



Boston BioChem  
6,000RSF

Tenant's Equipment  
120V Outlet for Tenant's  
Emergency Generator

Riverside Technology Center  
840 Memorial Drive  
Cambridge, MA

Floor Three  
Genetix Pharmaceuticals  
10,500RSF

**Floor Plan Exhibit**

Revised May 1, 2007

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**RIVERSIDE TECHNOLOGY CENTER  
LEASE EXTENSION AND MODIFICATION AGREEMENT  
TO THE LEASE BETWEEN**

**RIVERTECH ASSOCIATES II LLC AND GENETIX PHARMACEUTICALS, INC.**

This Lease Extension and Modification Agreement entered into this 24th day of November, 2009 by and between **Rivertech Associates II LLC**, a Massachusetts limited liability company with a principal address c/o The Abbey Group, 575 Boylston Street Boston, Massachusetts 02116, (the "Lessor"); and **Genetix Pharmaceuticals, Inc.**, with a business address at 840 Memorial Drive Cambridge, Massachusetts (the "Lessee"); relative to a certain Lease between Lessor's predecessor (Rivertech Associates LLC) and Lessee dated February 18, 2000 referred to herein as the "Original Lease" as amended by a certain Amended and Restated Lease Agreement dated May 18, 2007 (the "Lease Amendment") for certain office and laboratory space in the building at 840 Memorial Drive Cambridge, Massachusetts currently consisting of 10,500 rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building (the "Leased Premises"). The Original Lease, as amended by the Amended and Restated Lease Agreement, shall be referred to herein as the "Amended Lease".

**WHEREAS**, the Lessee desires to extend the Term of the Amended Lease, which is to expire on May 31, 2010, on terms and conditions agreeable to both Lessor and Lessee as a further modification to the Amended Lease, and Lessor assents to such extension of the Term by the Lessee on this basis;

**THEREFORE**, in consideration of One (\$1.00) Dollar and the other good and valuable consideration recited herein, effective and irrevocable as of the date hereof, the Lessor and Lessee hereby agree as follows:

**1. Modification to Original Lease/Extension of Term**

Lessee agrees to lease the Leased Premises commencing as of June 1, 2010 for an additional period of twelve (12) months, beginning June 1, 2010 (the "Extension Commencement Date") and ending on May 31, 2011 (the "Termination Date"); which additional period shall be referred to as the "Extended Term" or "Term".

Notwithstanding the commencement of the Extended Term on the Extension Commencement Date hereunder, this Lease Extension is to be considered a valid and binding obligation of the parties effective as of the date of execution, with the Amended Lease to continue to govern the Lessee's use and occupancy of the Leased Premises hereunder through the Term under the Amended Lease and up to the Extension Commencement Date hereunder. Thereafter, the Amended Lease (including amendment by this Lease Extension and Modification Agreement) shall conjunctively be and shall be referred to as the "Lease" as between the parties for the Extended Term.

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## **2. Terms And Conditions**

Lessee shall lease the Leased Premises commencing as of the Extension Commencement Date on the same terms and conditions of the Amended Lease, as modified by this Lease Extension and Modification Agreement, with exception only for those provisions as to which Lessor and Lessee have already performed their obligations as of the date hereof, (for example, Lessor has heretofore delivered the Leased Premises and Lessee has accepted the same).

## **3. Base Rent and Additional Rent**

Base Rent for each month of the Extended Term shall be Thirty Seven Thousand Six Hundred Twenty Five (\$ 37,625.00) Dollars per month, totaling Four Hundred Fifty One Thousand Five Hundred (\$ 451,500.00) Dollars for the twelve (12) month extension period.

In all instances Base Rent shall be payable in the corresponding monthly installments set forth above, due on the first of each month, in advance, and in all other respects shall be subject to the same provisions relating to Base Rent as set forth under the Original Lease.

In addition to Base Rent, Lessee shall continue to be responsible to pay all Additional Rent as set forth in Section 4 of the Amended Lease, consisting of Operating Expenses under Section 4A and Tax Expenses under Section 4B and Utilities under Section 4C of the Amended Lease; and all conditions thereunder are hereby restated and affirmed and shall govern the use and occupancy of the Leased Premises throughout the Extended Term.

## **4. Leased Premises in "AS/IS" Condition—No Defaults**

Lessee hereby acknowledges it is currently in possession of the Leased Premises and accordingly accepts the same for the Extended Term in its current "AS/IS" condition, without representation or warranty of any kind or nature arising from the extension of the Lease by Lessor and Lessee.

Lessor and Lessee each acknowledge that to the best of each of their respective knowledge, there are no material defaults by either presently existing under the Lease.

## **5. Brokers**

The parties hereby agree there are no brokerage or other third party fees or costs involved in this transaction and each agrees to indemnify, defend and hold harmless the other from and against any claims for brokerage fees, commissions or other such payments arising from this transaction.

## **6. Integration Of Documents: Supremacy**

The parties hereto intend that this Lease Extension and Modification Agreement operates to amend and modify the Amended Lease, and that those two documents shall be interpreted conjunctively; with any express conflict between the two to be resolved in favor of the stated terms of this Lease Extension and Modification Agreement. Except as modified hereby, all other terms and conditions of the Amended Lease shall remain unchanged and enforceable in a manner consistent with this Lease Extension And Modification Agreement.

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This Agreement shall be governed by the laws of the Commonwealth of Massachusetts. Any provisions deemed unenforceable shall be severable, and the remainder of this Lease Extension and Modification Agreement and the Original Lease shall be enforceable in accordance with their terms.

Witness our hands and seals as of the date first written above.

**LESSOR**

**RIVERTECH ASSOCIATES II, LLC**

By: /s/ Robert Epstein  
its duly authorized Manager

**LESSEE**

**GENETIX PHARMACEUTICALS, INC.**

By: /s/ Alfred E. Slanetz  
its duly authorized President/Vice President

By: Illegible Signature  
its duly authorized Treasurer/Ass't Treasurer

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**RIVERSIDE TECHNOLOGY CENTER**  
**SECOND LEASE EXTENSION AND MODIFICATION AGREEMENT**  
**TO THE LEASE BETWEEN**  
**RIVERTECH ASSOCIATES II LLC AND BLUEBIRD BIO, INC.**

This Second Lease Extension and Modification Agreement (the “Second Lease Extension Agreement”) entered into this 12<sup>th</sup> day of September, 2012 by and between **Rivertech Associates II LLC**, a Massachusetts limited liability company with a principal address c/o The Abbey Group, 575 Boylston Street Boston, Massachusetts 02116 (successor in interest as stated below, herein, the “Lessor”), and **Bluebird Bio, Inc.**, with a business address at 840 Memorial Drive Cambridge, Massachusetts (successor in interest as stated below, herein the “Lessee”), with respect to a certain Lease dated February 15, 2000 (as amended, as stated below) for certain office space in the building at 840 Memorial Drive Cambridge, Massachusetts.

WHEREAS, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain lease agreement dated February 18, 2000 (the “Original Lease”); and,

WHEREAS, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain Amended and Restated Lease Agreement dated May 18, 2007 (the “First Amended Lease Agreement”); and,

WHEREAS, Rivertech Associates II, LLC (as successor in interest to Rivertech Associates, LLC, the original signatory as Lessor under the Original Lease and First Amended Lease Agreement), and Genetix Pharmaceuticals, Inc., entered into a certain Lease Extension and Modification Agreement dated November 24, 2009 (the “First Lease Extension Agreement”); and,

WHEREAS, Rivertech Associates II, LLC and Bluebird Bio, Inc. (a Delaware corporation, formerly known as Genetix Pharmaceuticals, Inc.), entered into a certain Second Amended and Restated Lease Agreement dated October 19, 2010 (the “Second Amended Lease Agreement”); collectively, the Original Lease as amended and modified by the foregoing First Amended Lease Agreement, Lease Extension Agreement, and Second Amended Lease Agreement being referred to herein as the “Existing Lease”; and,

WHEREAS, the current Term under the Existing Lease expires on November 30, 2014 (the “Current Term”), and the Lessee seeks to extend the Current Term so as to expire on March 31, 2015, which date is referred to herein as the “Extended Term Termination Date”, and which entire term period as extended is referred to herein as the “Extended Term”; and,

WHEREAS, under the Existing Lease the Lessee leases and occupies approximately 9,488 rentable square feet of space located on the fourth (4<sup>th</sup>) floor of the Building, in addition to approximately fifty (50) rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building, for a total of approximately 9,538 rentable square feet of space in the Building, collectively known under the Existing Lease as the New Leased Premises and referred to herein as the “Existing Premises”; and,



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WHEREAS, by this Second Lease Extension Agreement Lessee seeks to add approximately 8,060 rentable square feet of space located on the fourth (4<sup>th</sup>) floor of the Building, in addition to approximately fifty (50) rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building, for a total of approximately 8,110 rentable square feet in the Building, as shown on Exhibit A hereto and collectively known as the “Expansion Space”; which, when added to the Existing Premises is collectively referred to herein as the “Total Leased Premises”; and,

WHEREAS, Rivertech Associates II, LLC and Bluebird Bio, Inc., seek by this current agreement to further amend and modify the Existing Lease to farther extend the Current Term of the Existing Lease, and to lease the Expansion Space, as set forth in detail below and under the terms and conditions of the Existing Lease as it is amended and modified hereby;

**THEREFORE**, in consideration of One (\$1.00) Dollar and the other good and valuable consideration recited herein, effective and irrevocable as of the date hereof, the Lessor and Lessee hereby agree as follows:

**1. Modification to Existing Lease / Extension of Current Term**

The Existing Lease expires on November 31, 2014 at the end of the Current Term. Lessee agrees to extend its tenancy as to the Existing Premises, commencing as of the end of the Current Term under the Existing Lease (i.e. from November 31, 2014), for an additional four (4) month period beginning on December 1, 2014 (the “Extension Commencement Date”) and ending on March 31, 2015 (the “Termination Date”); which additional period shall be referred to as the “Extended Term” or “Term”. Further, from the Expansion Space Delivery Dates (as defined herein) through the end of the Extended Term, Lessee also agrees to lease the Expansion Space (as defined herein) on the terms and conditions set forth herein.

Notwithstanding the commencement of the Extended Term on the Extension Commencement Date hereunder, this Lease Extension is to be considered a valid and binding obligation of the parties effective as of the date of execution of this Second Lease Extension Agreement by the parties and its approval by Lessor’s lender (which approval is an express condition to the extension and expansion contemplated herein and which Lessor shall seek immediately upon execution of this Agreement by both parties); with the Existing Lease to continue to govern the Lessee’s use and occupancy of the Existing Premises hereunder through the Term and up to the Extension Commencement Date hereunder, subject to the supplemental provisions hereof relating to the Expansion Space (defined herein). Lessee agrees to execute the Landlord’s lender’s standard Subordination Non-Disturbance and Attornment Agreement upon execution of this Second Lease Extension Agreement and Lessor agrees to promptly use commercially reasonable efforts to seek its lender’s execution of the same, but without any obligation to actually deliver the same to Lessee and without any material impact on this Second Lease Extension Agreement or the underlying Existing Lease upon any failure to do so.

**2. Terms and Conditions**

Lessee shall lease the Total Leased Premises (with the addition of the Expansion Space to be leased as of the Expansion Space Delivery Date) on the same terms and conditions of the Existing Lease, as modified by this Second Lease Extension Agreement, with exception only for

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those provisions as to which Lessor and Lessee have already performed their obligations as of the date hereof, (for example, Lessor has heretofore delivered the Existing Premises and Lessee has accepted the same). The Existing Premises is leased in the same "AS/IS" condition as it is as of the execution of this Second Lease Extension Agreement, and Lessee acknowledges Lessor is under no obligation to make any improvements or modifications thereto, in any manner.

### **3. Expansion Space Added to the Existing Premises**

Lessor shall deliver the Expansion Space to Lessee (to be added to and, in the aggregate, to constitute the Total Leased Premises). The Expansion Space shall be delivered to the Lessee upon Substantial Completion (as defined below) of Lessor's Work (as defined herein below) on the office portion of the Expansion Space (the "Office Expansion Space") and on the laboratory portion of the Expansion Space (the "Lab Expansion Space"), herein, the "Office Expansion Delivery Date" and the "Lab Expansion Delivery Date" respectively, and also referred to herein collectively as the "Expansion Delivery Dates". As of the respective Expansion Delivery Dates, the Office Expansion Space and the Lab Expansion Space, respectively, shall be Substantially Complete, vacated by any current tenants and occupants; with Lessor's Work having been performed in a good and workmanlike manner according to Lessor's Scope of Work; broom clean. All Lessee's Rent payments and other Lease obligations relating to the Expansion Space shall commence as of the Expansion Space Office Delivery Date and the Expansion Space Lab Delivery Date, respectively. All terms and conditions of the Lease shall govern the Lessee's use and occupancy of the Expansion Space as of the respective Expansion Space Delivery Dates.

Lessor's delivery of the Office Expansion Space and the Lab Expansion Space shall be evidenced in each instance by a written notice of delivery ("Lessor's Delivery Notice") given to Lessee on the actual date the respective portion of the Expansion Space is Substantially Complete and provided to Lessee for its occupancy. Lessee shall have five (5) business days to contest delivery if the Expansion Space is not Substantially Complete or it does not conform with the Lessor's Scope of Work and Lessor's Work by delivering its notice thereof in writing to Lessor; however, any listed items of a "punchlist" nature shall be agreed to by Lessor and Lessee and shall not be grounds to contest delivery, but nevertheless shall obligate Lessor to complete such punchlist items at the earliest practicable time under the circumstances.

The following conditions to the delivery of the Office Expansion Space and the Lab Expansion Space to the Lessee by the Lessor shall be met by the Lessor, at its sole cost and expense, prior to the respective Expansion Delivery Dates. The Lessor shall perform, at its sole cost and expense, such design and construction work as is necessary to deliver the respective portions of the Expansion Space to the Lessee in accord with the "Scope of Landlord's Work for Additional Premises Only" dated August 13, 2012 attached hereto as Exhibit A (the Exhibit referred to as the "Lessor's Scope of Work") and the design and construction obligations thereunder being referred to as the "Lessor's Work"). All components of Lessor's Work will be completed in accordance with all applicable laws, rules and regulations, including but not limited to the latest requirements of NFPA, ANSI Standards, ASHRAE Standards, National Electrical Code, Massachusetts State Building Code, and regulations of the City of Cambridge. Lessor shall deliver the Expansion Space with the base Building systems serving the same and with Lessee's specific mechanical, electrical and plumbing systems as required in Lessor's Scope of Work (i.e. Exhibit A hereto), in good operating condition and repair, and suitable for their intended uses.

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All utilities for the Expansion Space shall be in place and separately metered. The Building and the Expansion Space as delivered to the Lessee will be compliant with the Americans with Disabilities Act; NFPA compliant pursuant to the Massachusetts State Building Code; and with code compliant demising walls and common area corridors. Lessor shall provide Lessee with the environmental close-out report prepared by the former tenant for the Expansion Space, and said report shall not disclose any conditions as would materially impair Lessee's use of the Expansion Space. Subject to the foregoing, Lessor shall not be responsible for any other design or construction work with respect to either the Existing Premises under the Existing Lease, or the Expansion Space.

To the extent Lessee seeks to make any changes to the Lessor's Scope of Work as set forth on Exhibit A hereto: (i) such changes will be communicated in writing with sufficient specificity for Lessor to price the changes; (ii) Lessor will provide Lessee with pricing as to such changes and any estimated delays, if any, that may result for such changes; and (iii) in the event such changes affect the total cost of the Lessor's Scope of Work, Lessee shall either reimburse Lessor in advance for any increased costs and expenses, or Lessor shall credit Lessee for any savings against Lessee's next due payments of Rent hereunder. Upon receiving from Lessor the estimated cost and timing impacts of Lessee's proposed changes pursuant to subsection (ii) above, Lessee may choose to not move forward with such proposed changes provided it communicates its final intention to the Lessor in writing within ten (10) days of its receipt of Lessor's cost and timing impacts. Lessee shall be solely responsible for any delays in the completion of Lessor's Work arising from any such requested changes.

The Lessee shall be solely responsible, at its sole cost and expense, to perform such other specific design and construction work on the Expansion Space as it desires for its use and occupancy ("Lessee's Work"), upon completion of the Lessor's Work in the respective office and laboratory portions of the Expansion Space, and delivery of those respective portions the Expansion Space by the Lessor. Lessee shall be provided with access to the Expansion Space commencing upon execution of this Second Lease Extension Agreement, coordinated through the Lessor, for the purpose of performing preliminary work toward the installation of its equipment and wiring, provided such access and preliminary work does not materially interfere with Lessor's ability to perform and complete its Lessor's Work, which shall take precedence in all respects. Lessee's Work and all subsequent Lessee alterations to the Leased Premises that are performed by Lessee on or affecting the fire, life safety and/or sprinkler systems of the building shall be made in such a manner and under such conditions as to pose no adverse impact or interruption to such fire, life safety, and sprinkler systems, and so as not to delay, impair, or jeopardize the legal occupancy of other tenants in the Building as determined by Lessor and municipal fire and building inspection officials.

The Lessor's Work as to the Office Expansion Space and the Lab Expansion Space, separately, will be deemed "Substantially Complete" as to each: (a) when Lessor's Work is substantially complete in accordance with Lessor's Scope of Work as set forth in Exhibit A and the provisions of this Section 3, except for minor punch list items approved by Lessee that will not materially adversely affect Tenant's normal operations in said Expansion Space; Lessor's Work therein having been performed in a good and workmanlike manner with all necessary municipal approvals for occupancy.

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**4. Annual Base Rent and Additional Rent**

Annual Base Rent from the date of execution of this Second Lease Extension Agreement through the Extended Term, shall be as set forth below:

A. Annual Base Rent as applied to the Existing Premises  
(i.e. 9,538 rentable square feet of space)

- (i) Balance of the Current Term As set forth in the Existing Lease
- (ii) Extension Commencement Date through the Termination Date \$ 145,136.57 (\$ 36,284.14 / mo.)

B. Annual Base Rent as applied to the Expansion Space (i.e. 8,110 rentable square feet of space)

(i) Expansion Space Staggered Delivery Periods—Interim Rent/Per Diem

Lessor's estimated delivery of the Office Expansion Space is targeted to October 1, 2012. Lessor's estimated delivery of the Lab Expansion Space is targeted to December 1, 2012. The first day of the calendar month following the day on which the Office Expansion Delivery Date occurs is referred to herein as the "Expansion Rent Start Date". A per diem calculation is required to determine interim Rent obligations occurring upon delivery of the respective Office Expansion Space and Lab Expansion Space. Annual Base Rent for the total Expansion Space (office and laboratory) for the first full twelve month period following the Expansion Rent Start Date is \$395,362.50 (\$32,946.88 on a monthly basis). This computes to \$1,083.18 on a per diem basis. The per diem amount allocated to the Office Expansion Space is \$496.42, (i.e. 45.83% at 3,717 rentable square feet of a total 8,110 rentable square feet), the "Office Per Diem". The per diem amount allocated to the Lab Expansion Space is \$586.76 (i.e. 54.17% at 4,393 rentable square feet of a total 8,110 rentable square feet), the "Lab Per Diem".

(x) For the interim period between the Office Expansion Delivery Date and the Expansion Rent Start Date, the Annual Base Rent payment attributable to the Office Expansion Space shall be determined by taking the number of days from the Office Expansion Delivery Date through the Expansion Rent Start Date and *multiplying by* \$496.42. This amount shall be paid to Lessor.

(y) For the interim period between the Lab Expansion Delivery Date and the Expansion Rent Start Date (given the possibility the Expansion Rent Start Date may not have occurred due to delays in delivery of the Office Expansion Space), the Annual Base Rent payment attributable to the Lab Expansion Space shall be determined by taking the number of days from the Lab Expansion Delivery Date through the Expansion Rent Start Date (if applicable) and *multiplying by* \$586.76. This amount (if applicable) shall be paid to Lessor.

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(ii) Annual Base Rent due from the Expansion Rent Start Date through the next twelve (12) consecutive months is:

\$ 395,362.50 (\$ 32,946.88 /mo.)\*

\*[less the Lab Per Diem for each day in such period that the Lab Expansion Space is not delivered]

(iii) Annual Base Rent due through the next twelve (12) consecutive months (after (ii) above) is:

\$ 405,500.00 (\$ 33,791.60/mo.)

(iv) Annual Base Rent due through the next consecutive months (after (iii) above) to the end of the Extended Term is:

\$ *variable* (\$ 34,636.46 / mo.)

In all instances under A and B above, Annual Base Rent shall be payable in the corresponding monthly installments set forth above, due on the first of each month, in advance, and in all other respects shall be subject to the provisions relating to Annual Base Rent as set forth under the Existing Lease.

**C. Additional Rent.**

In addition to Annual Base Rent, Lessee shall continue to be responsible to pay all Additional Rent (Operating Expenses) under Section 3 of the Existing Lease, and all Additional Rent (Taxes) under Section 4 thereof, as applicable to both the Existing Premises and the Expansion Space, as invoiced by Lessor during the Extended Term. A current statement outlining the Operating Expenses incurred for the Building in 2011 is attached hereto as Exhibit B.

As the concept is used in the Lease to compute Additional Rent, Lessee's allocable pro rata share ("Allocable Percentage") shall be as follows:

(A) Allocable Percentage for the Existing Premises running through the end of the Term, shall be 7.4 %.

(B) Allocable Percentage for the Expansion Space, starting on the Expansion Space Delivery Dates and running through the end of the Term, shall be 6.29 %.

To the extent that the Expansion Space Commencement Dates do not fall on the first calendar day of a month, then the first month in which the Expansion Space Commencement Dates occur will have Additional Rent attributable to the Expansion Space prorated on a per diem basis for that month.

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**D. Rent and other Costs and Expenses.**

All Annual Base Rent, Additional Rent and other sums due as Rent shall be payable and in all other respects shall be governed during the remainder of the Current Term, and for the Extended Term, as contemplated under the Existing Lease, except to the extent modified and supplemented above. All other costs and expenses for utilities and services and attendant to operation of the Total Leased Premises (i.e. as applicable to both the Existing Premises, and to the Expansion Space as of the respective Expansion Space Delivery Dates), shall be borne by the respective parties as set forth in the Existing Lease.

**E. Security Deposit.**

The Security Deposit currently held by the Lessor shall continue to be held by Lessor during the Extended Term.

**5. Permitted Uses**

The Permitted Uses for the Total Leases Premises shall be office, R&D and laboratory use in conformity with all federal, state, municipal and local laws, codes, ordinances, rules and regulations of governmental authorities, committees, associations, or other regulatory committees, agencies or governing bodies having jurisdiction over the Total Leased Premises, and in conformity with the conditions and restrictions set forth in the Original Lease (which are hereby restated and affirmed by Lessee).

**6. Existing Premises in “AS/IS” Condition—No Defaults**

Lessee hereby acknowledges it is currently in possession of the Existing Premises and accordingly accepts the same from the date of this Second Lease Extension Agreement in its current “AS/IS” condition, without representation or warranty of any kind or nature arising from the extension of the Lease by Lessor and Lessee.

Lessor and Lessee each acknowledge that to the best of each of their respective knowledge, there are no material defaults by either presently existing under the Lease.

**7. Brokers**

The parties hereby agree there are no brokerage or other third party fees or costs involved in this transaction and each agrees to indemnify, defend and hold harmless the other from and against any claims for brokerage fees, commissions or other such payments arising from this transaction.

**8. Parking.**

Lessee shall be granted, at current rates (which may be increased from time to time to reflect market increases), the right (but not the obligation) to park up to twenty seven (27) cars in total in the Building’s on-site indoor parking lot or facility on an unassigned and unreserved basis, in single or tandem spaces or on a valet basis which Lessor in its sole discretion shall designate from time to time. The initial parking rate therefor shall be \$225 per month, per car, which monthly rate may be changed by Lessor in its discretion subject to and reflective of periodic

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market changes. Additionally, Lessee shall be entitled to rights to park additional cars in the Building garage (but subject to availability as determined by Lessor, and only on a valet basis, and then only to the extent Lessor is providing valet service to the Building garage which Lessor shall not be obligated to do), at then current rates as set by Lessor in its discretion. If Lessor cannot accommodate Lessee's needs for additional parking in the Building garage, then Lessor will assist Lessee in identifying alternative off-site parking, but Lessor shall bear no liability nor shall it be deemed any default under the Lease if such additional Building garage parking or off-site parking do not materialize. All payments for these parking rights shall be considered to be Additional Rent under this Lease. This provision supersedes any contrary provisions of the Existing Lease and the specific numeric rights set forth above supplant the numeric rights otherwise set forth in the Existing Lease.

#### **9. Lessee's Option to Extend**

Provided Lessee has not defaulted (after applicable notice, grace and cure periods, if any) under the Lease from the date hereof forward more than two (2) times, and further provided no uncured default on the part of Lessee is then existing, Lessee shall have the option to further extend the Term of this Lease as to the Total Leased Premises (i.e. inclusive of the Expansion Space) on the terms and conditions herein, for one additional period of thirty six (36) months (herein, the "Additional Term Extension Period") at the then current "Market Rent" (including annual escalations thereon for each year of the extended term based on increases in the Consumer Price Index or fixed increases, as the case may be, as determined by then prevailing market forces), but no less than an amount equal to the annualized Rent per rentable square foot of Total Leased Premises space as of the final full month of the last Lease Year hereunder (the "Extension Rent Floor"). Said Additional Term Extension Period shall commence, subject to proper exercise of Lessee's option hereunder, at the end of the Extended Term (i.e. at the end of March, 2015) and shall terminate on that date which is thirty six (36) consecutive months thereafter (i.e. March 31, 2018). Lessee shall exercise its option by delivering to Lessor its written notice not later than ten (10) full months (but not sooner than twelve (12) full months) prior to the end of the Extended Term. Once delivered, written notice to extend is irrevocable.

"Market Rent" as used herein, shall be that rent charged for comparable research laboratory and office space of similar age and condition in laboratory buildings the mid-Cambridge submarket as of the end of the Extended Term. If, after good faith attempts prior to the expiration of the original Term, the Lessor and Lessee cannot agree on a figure representing Market Rent, then either party, upon written notice to the other, may request appraisal and arbitration of the issue as provided in this section. Within fourteen (14) days of the request for arbitration, each party shall submit to the other the name of one unrelated individual or entity with proven expertise in the leasing of commercial real estate in greater Boston/Cambridge to serve as that party's appraiser. Each appraiser shall be paid by the party selecting him or it. The two appraisers shall each submit their final reports to the parties within thirty (30) days of their selection making their determination as to Market Rent (subject however, to the Extension Rent Floor). The two appraisers shall meet within the next fourteen (14) days to reconcile their reports and collaboratively determine the Market Rent. They shall each make their determination in writing (subject however, to the Extension Rent Floor), including a statement if such is the case, that they are at an impasse. Such a statement of impasse shall be submitted to the parties along with the Market Rent figure which each appraiser has selected and his reasons and substantiation

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therefor. The appraisers, in case of an impasse, shall also agree on one unrelated individual or entity with expertise in commercial real estate in greater Boston, who shall evaluate the reports of the two original appraisers and within fourteen (14) days of submission of the issue to him, make his own determination as to a figure representing Market Rent (subject however, to the Extension Rent Floor). The determination of this individual or entity (i.e. arbitrator) absent, fraud, bias or undue prejudice shall be binding upon the parties.

Annual Base Rent and Additional Rent during any Additional Term Extension Period shall be payable in advance, in equal monthly installments on the first day of each calendar month.

Lessee, in addition to the sums payable annually to Lessor as Annual Base Rent, shall pay to Lessor for each year of the Additional Term Extension Period, as Additional Rent, Lessee's Allocable Percentage (as determined by the approximate total rentable space leased) for Operating Expenses, Real Estate Taxes and utilities as contemplated in Section 4 hereof.

**10. Access; Elevators; Dumpsters; Signage**

Lessee shall have 24/7 access to the Total Leased Premises (with regard to the Expansion Space, such access shall be allowed following the applicable Expansion Delivery Dates), the common areas and elevators serving the Total Leases Premises, the freight elevators servicing the Building, the loading docks servicing the Building and the dumpster and/or compactor servicing the Building.

Lessor shall provide, at Lessor's expense, building standard signage in the Building's lobby, in any Building directory, and at the main entrance to the Total Leased Premises.

**11. Integration of Documents; Supremacy**

This Second Lease Extension Agreement contains the full understanding and agreement between the parties. The parties hereto intend that this Second Lease Extension Agreement operates to amend and modify the Existing Lease, and that those documents shall be interpreted conjunctively; with any express conflict between the two to be resolved in favor of the stated terms of this Second Lease Extension Agreement. Except as modified hereby, all other terms and conditions of the Existing Lease shall remain unchanged and enforceable in a manner consistent with this Second Lease Extension Agreement. Defined terms used in this Second Lease Extension Agreement that are not otherwise defined herein shall have the definitions ascribed to such terms in the Existing Lease.

This Agreement shall be governed by the laws of the Commonwealth of Massachusetts. Any provisions deemed unenforceable shall be severable, and the remainder of this Second Lease Extension Agreement and the Existing Lease shall be enforceable in accordance with their terms.

**[Signature Pages Follow]**



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Witness our hands and seals as of the date first written above.

**LESSOR**

**RIVERTECH ASSOCIATES II, LLC**

By: /s/ Robert Epstein  
its duly authorized Manager

**LESSEE**

**BLUEBIRD BIO, INC.**

By: /s/ Nick Leschly  
its duly authorized President/Vice President

By: /s/ Jeffrey T. Walsh  
its duly authorized Treasurer/Ass't Treasurer

**See Attached**

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**BLUEBIRD BIO**

Floor Four  
840 Memorial Drive  
Cambridge, MA

AUGUST 13, 2012

**SCOPE OF LANDLORDS WORK FOR ADDITIONAL PREMISES ONLY**

\*\*\* Space will be emptied of all furnishing prior to move-in date

**Partitions:** Partitions are to be removed and relocated as indicated on the accompanying plans. Existing and new walls are comprised of gwb over steel studs and extend from floor to the underside of the suspended ceiling. The walls demising the Administrative areas from R&D areas extend to the underside of the deck above. All walls will be finished with two coats water based paint. The entire office space will have 2 new coats of water based paint to match bluebird's existing space color scheme.

Existing "lavatory" room will have plumbing removed and remain as storage room. New storage room will have VCT removed and new matching carpeting.

**Glass Panels:** Where they exist, glass panels to remain. Glass panels from 4131 and 4130 will be removed to allow for the respective doors to be relocated. This is to allow for the modern-fold partition to be stored on the wall in between the doors when it is not separating 4131 and 4130.

**Entry:** Existing glass entry to remain.

**Doors:** Existing doors will be reused and relocated as necessary provided they have existing full lite glazing. All hardware are lever handles with brushed stainless finish. New office doors will be installed with full lite glazing where applicable. All office doors will have full lite glazing. Office and conference room door hardware will be comprised of passage function latchsets with brushed stainless finish. Storage room latchsets will be comprised of class room function latchsets with brushed stainless finish.

**Floors:** All carpeted areas in the administrative which are affected by construction will be re-carpeted with matching Shaw Contract nylon loop carpet from "Turn Key Collection. 4" vinyl cove base has been installed at intersection of walls and carpet/vct. Vinyl tile installed in the floor of the kitchenette to remain. VCT will be removed from the storage area and be replaced with matching carpeting, 4" vinyl cove base will be installed in the storage areas as well as areas affected by construction.

**Ceilings:** All existing ceiling tiles will remain or be replaced as necessary. Any damaged ceiling tiles will be replaced with matching ceiling tiles. Any damage to ceiling grid will be repaired.

**Lighting:** All existing fluorescent lights will remain and be relocated as necessary to accommodate the new configuration. All existing fluorescent lights will be inspected to insure proper functioning and be repaired or replaced as necessary.

BluebirdBio Final Scope of Work

8/13/2012

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HVAC: The base building HVAC distribution system will be inspected and adjusted as necessary to assure distribution, airflow, and proper operation of thermostats and variable air volume (VAV) boxes. There will be a redistribution of the system to accommodate 5 separate zones as indicated on the drawings. This work will be designed by landlord's consulting engineer. The office area will be provided with make-up air from either the 30 ton roof mounted MUA unit or house systems in order to provide fresh air to the office and maintain positive pressure with respect to R&D space. A pre-construction balancing/CFM reading will be done to show existing air balance in the space. The landlord's engineer will perform HVAC load calculations to take into account insulation, square footage, occupancy and equipment heat loss to determine heating and cooling loads. Expected equipment and occupant load shall be provided to the landlord's engineer for this purpose. The space will be balanced according to these calculations. All balancing will be conducted by a NEEB certified balancing technician. Pre-construction balancing, engineering calculations and post-construction "as built" documentation/balancing reports will be provided to the tenant or the tenant's designee. The new space will conform to ASHRAE Standard 55 "Thermal Environmental Conditions for Human Occupancy". All HVAC equipment shall be inspected by the landlord and replaced as necessary. The lessee accepts the existing HVAC system provided that it is in good working order, that it conforms to ASHRAE Standard 55, and that a reasonable temperature is maintained in the office areas. In the event during the lease term the HVAC system does not meet the aforementioned prerequisites the landlord will provide a new system which will be replaced at the Landlord's cost. The tenant will be responsible for maintaining and repairing all tenant specific supplemental mechanical equipment beyond the 6 month warranty period. Tenant specific mechanical equipment will be delivered in good operating condition.

Kitchenette: The kitchenette will remain undisturbed.

Electrical: Existing electrical outlets throughout the administration area and the office space in the R&D area in the form of existing and new 110v outlets to remain. All utilities servicing the tenant's premises and equipment are separately metered and will be read monthly by the landlord for reimbursement by the tenant. New outlets will be installed to accommodate the new layout. Each office will have a minimum of 3 120/20a 5-20R receptacles. All electrical circuits will be labeled via labels at the outlet, junction box, safety switch, or other corresponding electrical equipment and a corresponding label on the electrical panel indicating the appropriate circuit breaker.

Furnishings: No reception desk cubicles, work stations or furniture of any sort shall be provided by the landlord.

Specialties: A demountable partition by Modernfold or equal will be installed as indicated in the plans. This wall will have a "Dry Erase" surface from floor to ceiling. BluebirdBio or their designee will have access to the space during the renovation to inspect progress and that the work being done conforms to this scope.

Fire Protection: Fire protection will be added as needed and required by Massachusetts Building code, NFPA and local Fire Code.

Work included in this scope will comply with the Massachusetts Building Code and NFPA.

BluebirdBio Final Scope of Work

8/13/2012

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## **R&D AREA**

Demolition: Existing lab benches, plumbing, piping, vinyl tile, doors and walls required to be removed to accommodate new plan will be removed. The existing 6' hood shall be capped and removed from the premises. The existing wing wall adjacent to the hood shall be removed per attached drawings.

The entire R&D area will have 2 coats water based paint applied per Bluebird bio's existing paint scheme

New Partitions: New partitions enclosing new tissue culture suites shall consist of existing walls comprised of 5/8" gwb over 3 1/2" steel studs and extending from floor to 6" above the underside of the suspended ceiling. Walls and trim will be finished with two coats water based paint. Paint finish in tissue culture rooms to be semi gloss.

The tissue culture rooms will be separated from the main laboratory by a hallway as indicated in the drawing.

Existing glass panels in the exterior offices shall be removed.

The walls surrounding the "radio-isotope room" shall extend to the underside of the deck above and have an additional layer of 5/8" gwb applied to each accessible side of the wall. Walls will be constructed to the deck above with 2 layers of 5/8" GWB. Walls will be completely sealed with appropriate sealant in order to prevent any contamination of adjoining spaces. Work will be consistent with attached drawing and keynotes.

The existing exterior window in the office to become the darkroom shall be blacked out via either applied opaque vinyl or an opaque panel which will prevent any light from entering via the window.

Doors: Where indicated on the drawings, Horton Ultra-Clean Series Atmospheric 110 doors with push plate activators and safety scan sensors will be installed. Buttons to activate the doors will be installed.

A 36" diameter circular darkroom door shall be installed in the office to become the darkroom as indicated on the plans. The door shall fit within a 36" wall opening and have a "pop-out" feature with breakaway hardware for use in emergencies and shall conform to Massachusetts Building Code requirements.

Floors: All new tissue culture suite floors will be covered with seamless vinyl with integral vinyl base. Floors where noted in the drawing as indicated by keynotes, to include the radio-isotope room and hallway, will be covered with seamless vinyl with integral vinyl base. The damaged vinyl floor in the autoclave room will be repaired.

Ceilings: Except for the tissue culture suite, existing building standard ceiling tiles will remain in the existing grid. In the Tissue Culture Suites, and as indicated in attached drawing keynotes, to include the radio isotope room and hallway, new solid surface washable ceiling tiles will be installed in the new ceiling grid with vinyl gasketing. Any damaged ceiling tiles will be replaced. Any damage to ceiling grid will be repaired.

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Lighting: Except for the new Tissue areas, existing 2x4 and 2x2 fluorescent lights will remain. Solid surface lens type fluorescent lights will be used in the Tissue Culture, radio-isotope, and Glasswash/Autoclave rooms.

BENCHES: The three remaining lab benches with sink will remain. Oak benches with stainless steel sinks and acid resistant plastic laminate tops will be installed in the two new tissue culture suites while the existing bench and sink in the existing tissue culture suite shall be relocated as indicated on the plans. Oak benches/casework with a stainless steel sink and epoxy resin counter top will be installed in the radio-isotope room in the form of an L-shape as indicated in the attached drawing. The sink in the radio-isotope room will have a motion activated faucet. The casework and countertop in the radio-isotope room will be designed such that space for a 4' fume hood or BSC be provided for future installation. Each lab sink will have protected hot and cold water and deck mounted emergency eyewash stations.

HVAC: The base building HVAC distribution system will be inspected and adjusted as necessary to assure good operating condition and repair, distribution, airflow, and proper operation of thermostats and variable air volume (VAV) boxes. The existing supplemental air conditioning units will be inspected and put in good operating condition and repair. One of these supplementary systems will service the main laboratory (4114), the second will service the existing tissue culture suite 4118. The third existing unit will service the new tissue culture suite (4119) or suite's (4119 and 4120) depending on HVAC load requirements. If necessary a new unit with integral electric heat will serve the tissue culture suite (4120). The air conditioning units should correspond to what HVAC heating and cooling is required, as indicated by analysis conducted by the landlord's engineer. All tenant specific mechanical systems shall be warranted for proper operation for a period of six months provided tenant enters into an appropriate preventive maintenance agreement for this equipment as stated below. Tenant specific mechanical equipment will be delivered in good operating condition. A pre-construction balancing/cfm reading will be done to show existing air balance in the space. The landlord's engineer will perform load calculations to take into account insulation, square footage, occupancy and equipment heat loss to determine heating and cooling loads. Expected equipment and occupant load shall be provided to the landlord's engineer for analysis and design of the HVAC system. The space will be balanced according to these calculations. All balancing will be conducted by a NEED certified balancing technician. Pre-construction balancing, engineering calculations and post-construction "as built" documentation/balancing reports will be provided to the tenant or the tenant's designee. The new space will conform to ASHRAE 55 "Thermal Environmental Conditions for Human Occupancy" and ASHRAE Standard 62.1 2010 "Ventilation for Acceptable Indoor Air Quality". All HVAC equipment shall be inspected by the landlord and replaced as necessary to ensure good operating condition and repair. The lessee accepts the existing HVAC system provided that it is in good working order, that it conforms to ASHRAE Standard 55, and 62.1 2010, and that a reasonable temperature is maintained in the R&D areas. In the event during the lease term the HVAC system does not meet the aforementioned prerequisites the landlord will provide a new system which will be at the Landlord's cost. Tissue culture spaces will have 6 to 15 air changes per hour. Spaces will be pressurized according to the attached drawing.

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The fume hood which is to remain in the laboratory is connected to a roof mounted exhaust fan and shall be air balanced to assure a minimum of 100 FPM airflow across the sash at an 18" sash opening. Fume hood will be equipped with a flow monitoring device which will indicate high/low limits. The remaining approximately 800cfm of exhaust shall be connected to ceiling grilles in the main lab and shall exhaust the lab. If more CFM is required to exhaust the lab according to the attached pressurization drawing it will be provided at the Landlord's expense. Exhaust must create negative pressure in the hallway connecting the tissue culture suites as well as create a negative with regard the office and main lab. The landlord's engineer will make the necessary calculations and resulting balancing recommendations to ensure that the space is pressurized accordingly. Make up air shall be provided from a 30 ton roof mounted make-up air unit and shall be fed to each of the three tissue culture suites (4118, 4119, and 4120) and the "radio-isotope room" (4116). Make up air shall also be provided to the main lab space (4114). All air supplied to each of the tissue culture suites shall be via 95% HEPA filters with 30% pre-filters on each of the ac units, MUA units, feeding, the spaces. Each tissue culture suite shall have a pressure relief exhaust grill installed on a wall adjacent to the main lab. The tenant will be responsible for maintaining and repairing all tenant specific supplemental mechanical equipment beyond the 6 month warranty period.

The Lab/Office will be pressurized as indicated in the attached drawing. Room for a 4' wide fume hood or ducted BSC will be left in the Radio-Isotope Room. The cost of operating and maintaining both exhaust fan and make up air unit shall be a tenant expense. All Tenant-specific mechanical equipment shall be put on a preventive maintenance agreement by the tenant and at Tenant's expense for the duration of the Lease.

Plumbing and Waste: The main cold water supply to the lab is located in the "Lab support" room which also contains a hot water heater, a water check meter and a backflow prevention device. All lab waste is contained in polyethylene piping and leads to an acid waste system consisting of a tank with limestone chips located in an accessible location on the third floor directly below the premises. Lab waste and holding within the "isotope room" will be connected to the existing laboratory waste system. Before the room is used for radio-isotopes this drain will be disconnected and is subject to future engineering and installation by the tenant. Future modifications to this drain is not subject to this scope of work. The laboratory wastewater system will be maintained by the tenant who shall also be responsible for required municipal discharge permits, sampling and testing.

Safety Showers will be added in rooms 4116 4119 and 4120 and will meet or exceed ANSI standards. Requirements for safety showers and eyewash stations throughout the lab space will be accessed by the landlord and will also be made to comply with ANSI standards.

Autoclave Room: The landlord has provided a room in which an autoclave can be installed. Actual equipment will be provided by the tenant. The existing laboratory sink with protected hot and cold water and eyewash shall remain. The seamless vinyl floor with integral base shall be repaired as required. Electrical hot water, drain will be installed to accommodate the future installation of a dishwasher.

Electrical: Power to various locations within the laboratory exists in the form of existing 110v and 208v outlets. 12 additional 120v/20A circuits, 7 additional 220v/20A circuits shall be added along with a new electrical panel will be connected to the existing roof mounted back up generator. In addition, one existing outlet shall be placed on backup power as well as the 2hp

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motor driving the roof mounted exhaust fan. Location of these outlets are as indicated on the accompanying plans. Tenant shall share the cost of maintenance and repairs of the back up generator with any other tenants who are also connected to it. All electrical power, natural gas and water to the tenant's premises and equipment will be separately metered and read monthly by the landlord for reimbursement by the tenant. New standby power outlets will be added as indicated on attached drawing. New normal power outlets will be added as indicated on attached drawing. All electrical circuits will be labeled via labels at the outlet, junction box, safety switch, or other corresponding electrical equipment and a corresponding label on the electrical panel indicating the appropriate circuit breaker.

CO2 Piping: Piping for tenants CO2 shall be installed as indicated in the accompanying drawings. These shall consist of 1/2" copper piping from tenant's existing "tank farm", 20 CO2 drops to 1/2" ball valve to 1/4" hose barbs as indicated, 16 brass 1/2" ball valve shut-offs and 5 above ceiling capped 1/2" copper lines for future use. Gas regulators, if required, shall be provided by the tenant.

Vacuum lines will be installed as shown on the attached drawing. Vacuum drops will consist of 1/2" type L copper piping from the house vacuum system to drops as indicated on the attached drawing. Each drop will consist of 1/2" copper pipe to 1/2" ball valve to 1/4" hose barb.

BluebirdBio or their designee will have access to the space during renovation to inspect the progress and that the work being done conforms to this scope.

Fire Protection: Fire protection will be added as needed and required by Massachusetts Building Code, NFPA and local Fire Code.

Work included in this scope will comply with both the Massachusetts Building Code and NFPA.

Laboratory Safety Showers/Eyewash stations will be installed as required per ANSI standards.

Work will be conducted as indicated by attached drawing keynotes.

Scope of Work to include all Keynotes in attached drawings with the exception of MEPs in the Radio Isotope Room.

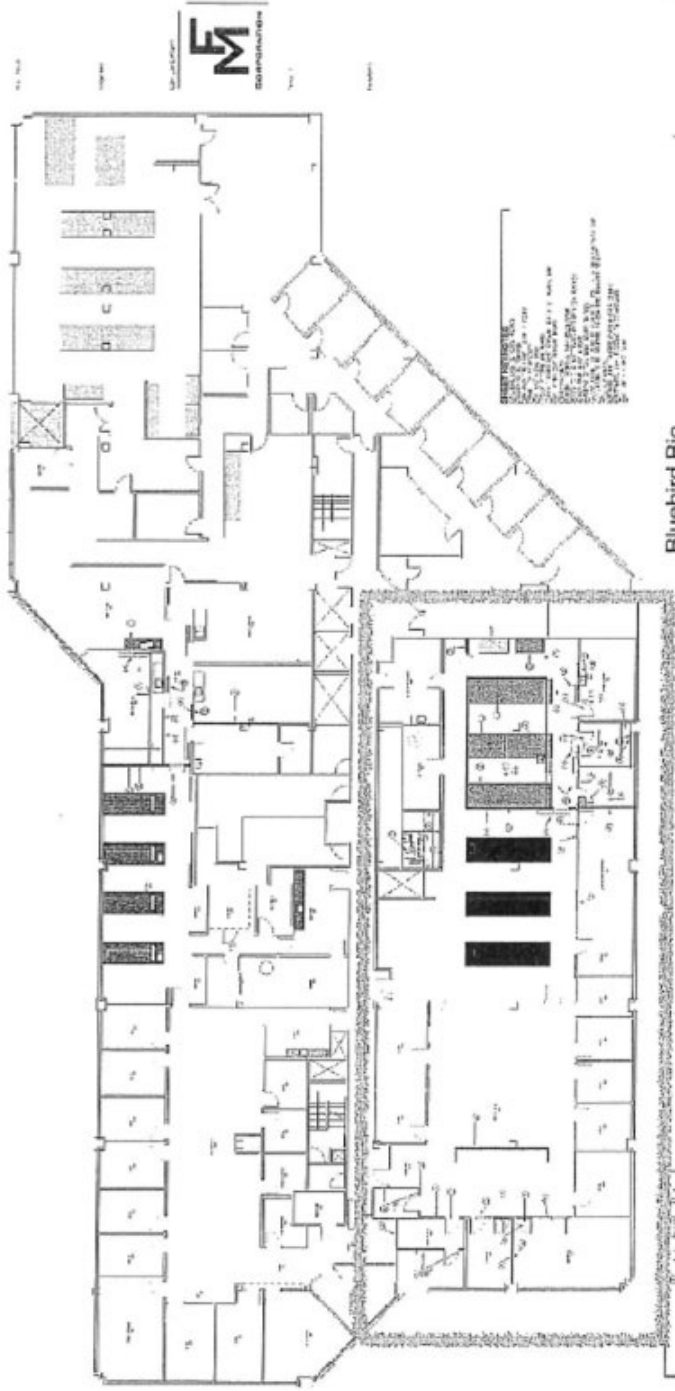
Drawings included in this scope of work are diagrammatic in nature. All pre-existing construction and new construction will be reviewed by the landlord's architect and engineer to insure indicated changes are made in accordance with Massachusetts Building Code and the NFPA.

BluebirdBio Final Scope of Work

8/13/2012





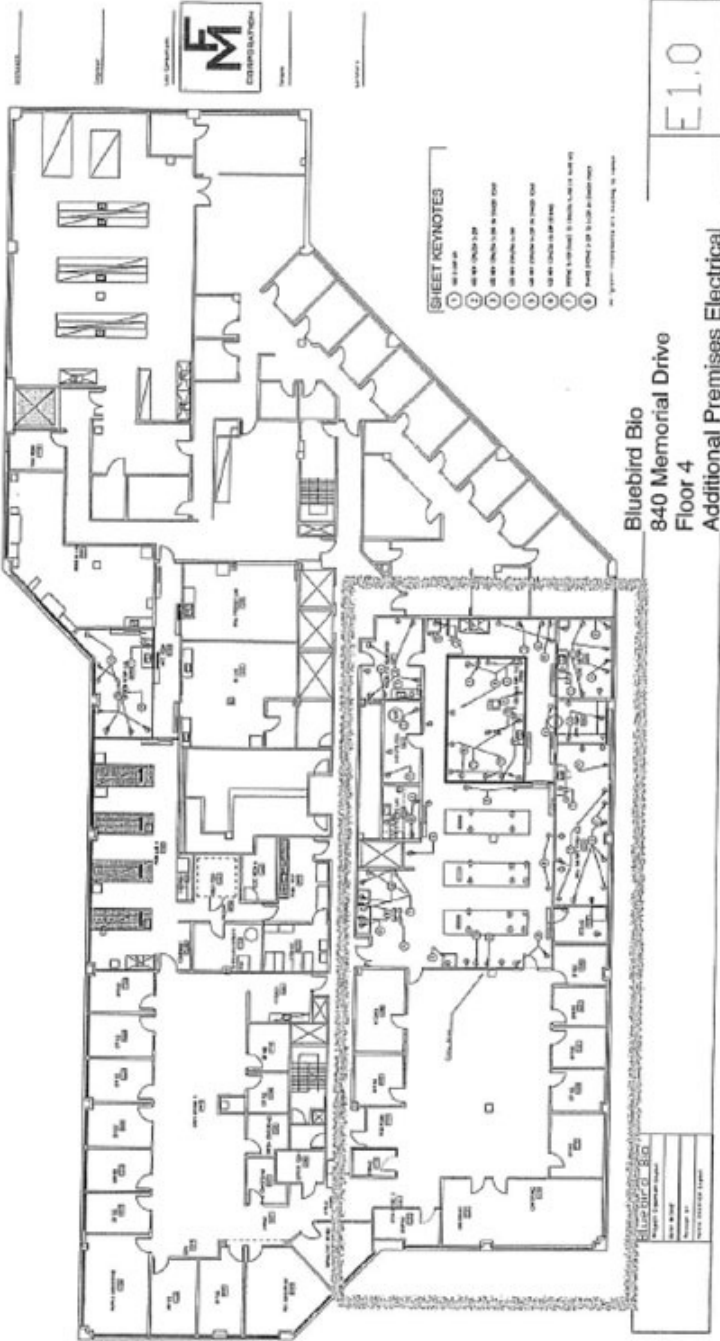


Bluebird Bio  
 840 Memorial Drive  
 Floor 4  
 Additional Premises Plan Revision

DATE: 11/11/2014  
 DRAWN BY: [Name]  
 CHECKED BY: [Name]

40

REVISIONS:  
 1. 11/11/2014: [Description]  
 2. 11/11/2014: [Description]  
 3. 11/11/2014: [Description]  
 4. 11/11/2014: [Description]  
 5. 11/11/2014: [Description]



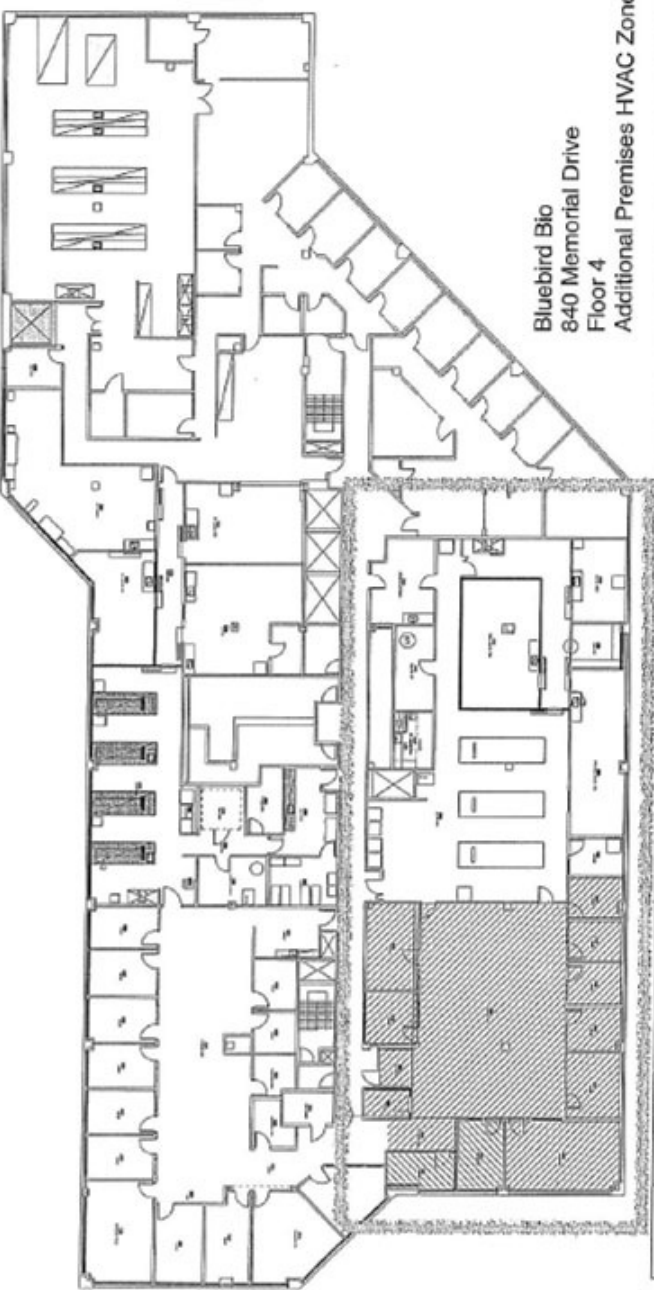
- SHEET KEYNOTES**
- 1. SEE SHEET E1.1
  - 2. SEE SHEET E1.2
  - 3. SEE SHEET E1.3
  - 4. SEE SHEET E1.4
  - 5. SEE SHEET E1.5
  - 6. SEE SHEET E1.6
  - 7. SEE SHEET E1.7
  - 8. SEE SHEET E1.8
  - 9. SEE SHEET E1.9
  - 10. SEE SHEET E1.10

Bluebird Bio  
 840 Memorial Drive  
 Floor 4  
 Additional Premises Electrical

E1.0



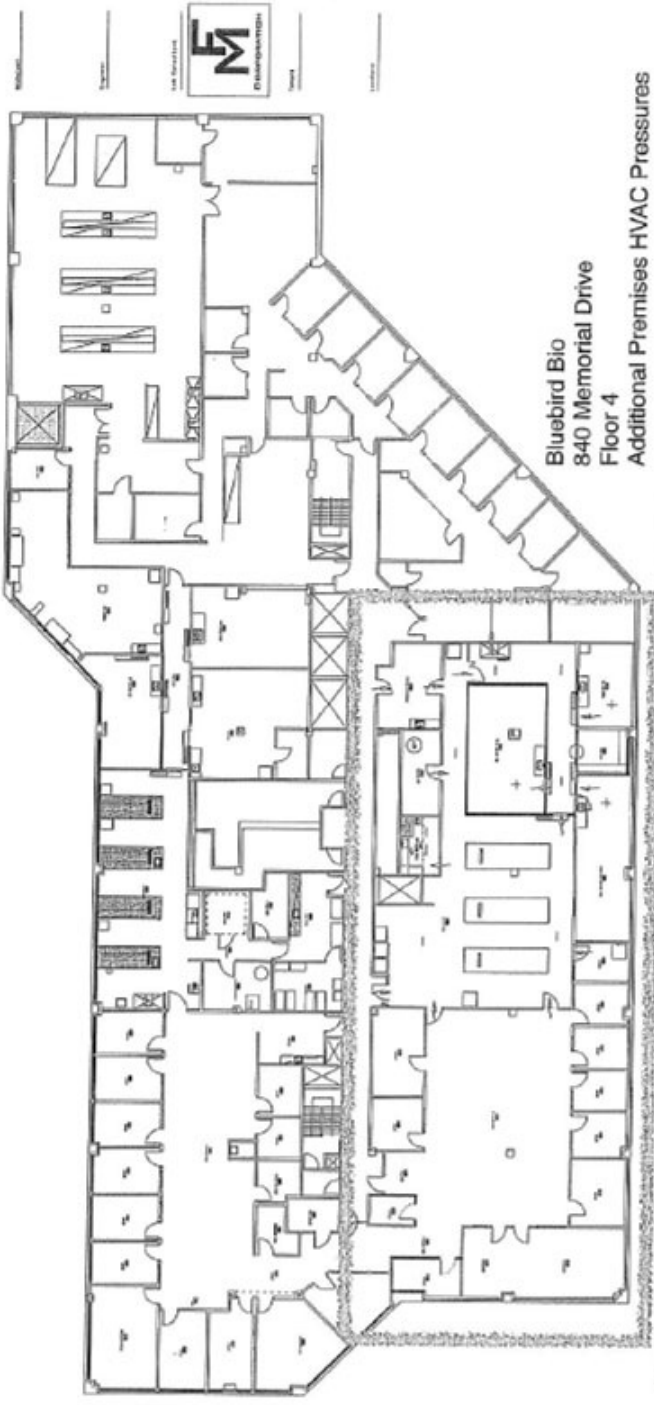
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| Project: Bluebird Bio |
| Sheet: E1.0           |
| Date: 10/15/2019      |
| Drawn by: [Name]      |
| Checked by: [Name]    |



Bluebird Bio  
840 Memorial Drive  
Floor 4  
Additional Premises HVAC Zones

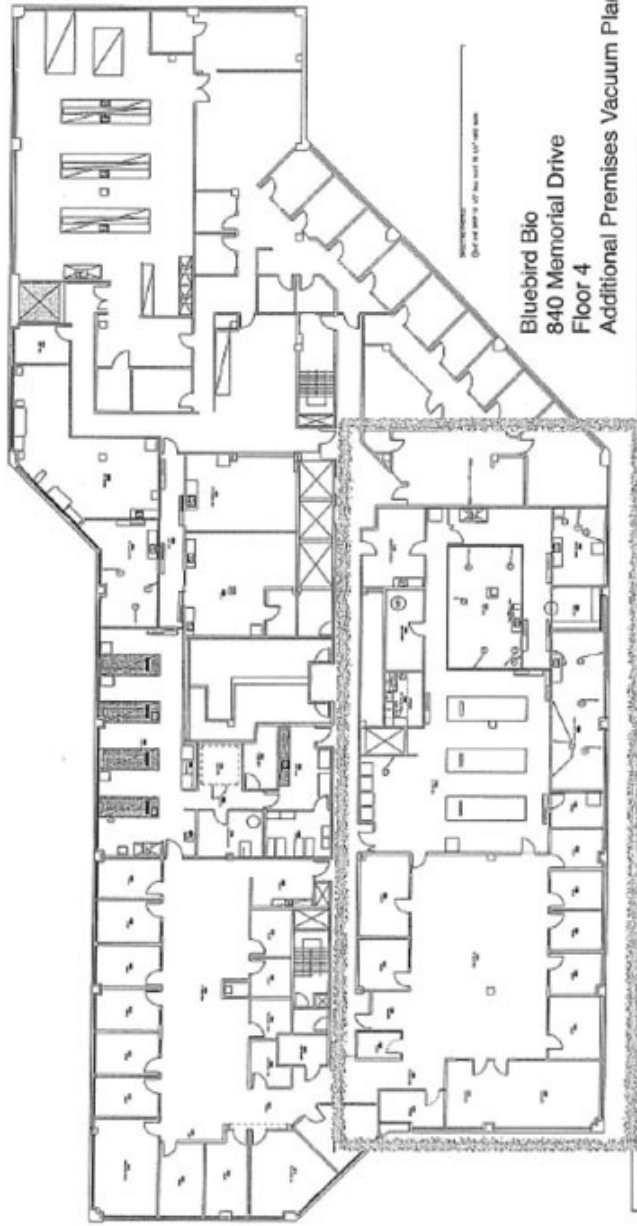
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| Bluebird Bio                   |
| 840 Memorial Drive             |
| Floor 4                        |
| Additional Premises HVAC Zones |

H1.0



|              |
|--------------|
| Bluebird Bio |
| Project Name |
| Date         |
| Scale        |
| Sheet No.    |

H1.1

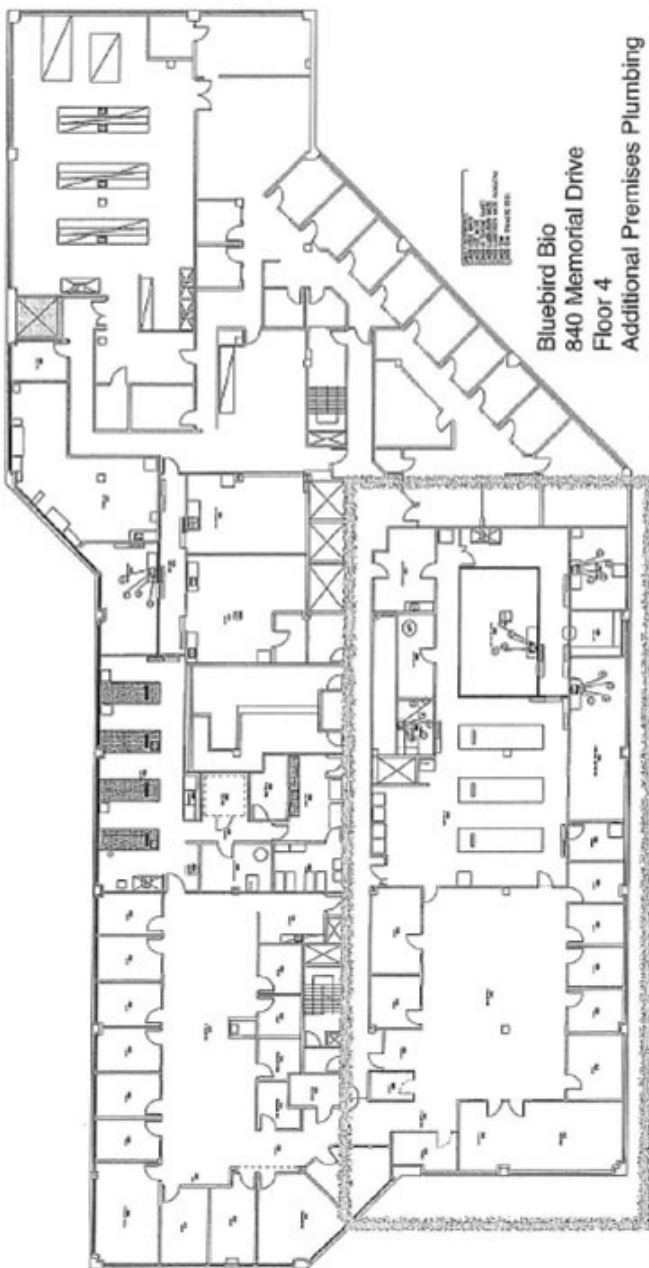


NOT TO SCALE  
DATE: 08/20/18  
SCALE: 1/8" = 1'-0"

**Bluebird Bio  
840 Memorial Drive  
Floor 4  
Additional Premises Vacuum Plan**

|                      |              |
|----------------------|--------------|
| Project Name         | Bluebird Bio |
| Project No.          | 18-001       |
| Revision No.         | 01           |
| Revision Description | Final        |

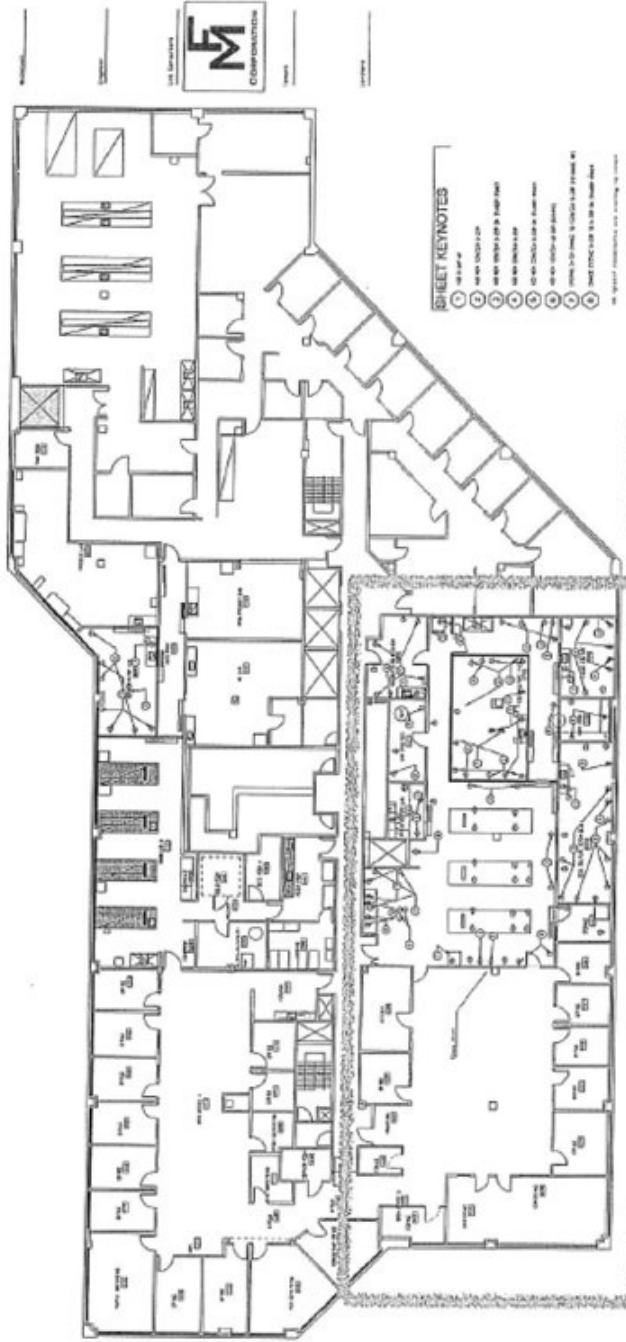
P1.1



Bluebird Bio  
840 Memorial Drive  
Floor 4  
Additional Premises Plumbing

Bluebird Bio  
Project Location: \_\_\_\_\_  
Date: 8/20/20  
Revision: \_\_\_\_\_  
Sheet: \_\_\_\_\_

P1.3



- SHEET KEYNOTES**
- 1. SEE PLAN
  - 2. SEE SCHEDULE
  - 3. SEE SCHEDULE
  - 4. SEE SCHEDULE
  - 5. SEE SCHEDULE
  - 6. SEE SCHEDULE
  - 7. SEE SCHEDULE
  - 8. SEE SCHEDULE

Bluebird Bio  
 840 Memorial Drive  
 Floor 4  
 Additional Premises Electrical

|                    |              |
|--------------------|--------------|
| Project Name:      | Bluebird Bio |
| Sheet No.:         | E1.0         |
| Revision:          |              |
| Total Sheet Count: |              |

E1.0



**See Attached**

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**EXHIBIT B—Operating Expenses 2011**

840 Memorial Drive—Riverside Technology Center

| <u>DESCRIPTION</u>                 | <u>Total</u>      | <u>PSF</u>    |
|------------------------------------|-------------------|---------------|
| HEAT                               | \$ 36,863         | \$0.28        |
| BUILDING ELECTRIC                  | \$152,969         | \$1.18        |
| WATER & SEWER                      | \$ 30,053         | \$0.23        |
| ELEVATOR MAINTENANCE               | \$ 19,295         | \$0.15        |
| PARKING/CAFÉ EXPENSE               | \$ 26,525         | \$0.20        |
| RUBBISH REMOVAL                    | \$ 17,104         | \$0.13        |
| INSURANCE                          | \$ 30,234         | \$0.23        |
| GROUNDS CARE                       | \$ 19,459         | \$0.15        |
| LEGAL/ACCT/ADMIN                   | \$ 18,675         | \$0.14        |
| JANITORIAL SERVICES                | \$ 47,707         | \$0.37        |
| GENERAL MAINTENANCE                | \$ 63,172         | \$ 0.49       |
| HVAC MAINTENANCE                   | \$ 47,827         | \$0.37        |
| LIFE SAFETY SYSTEMS                | \$ 29,593         | \$0.23        |
| MANAGEMENT*                        | \$280,302         | \$2.16        |
| <b>Real Estate Taxes (FY 2011)</b> | <b>\$ 659,946</b> | <b>\$5.10</b> |

\* Based upon 5% of income; but not less than this amount.

Tenant's Applicable Percentage is as follows:

As to the Leased Premises: 13.69%.

Existing Premises: 7.40%

Expansion Premises: 6.29%

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**RIVERSIDE TECHNOLOGY CENTER**  
**THIRD AMENDED LEASE AGREEMENT**  
**BETWEEN**

**RIVERTECH ASSOCIATES II LLC AND BLUEBIRD BIO, INC.**

This Third Amended Lease Agreement (the "Third Amended Lease Agreement") entered into this 29 day of July 2013 by and between **Rivertech Associates II LLC**, a Massachusetts limited liability company with a principal address c/o The Abbey Group, 575 Boylston Street Boston, Massachusetts 02116 (successor in interest as stated below, herein, the "Lessor"), and **Bluebird Bio, Inc.**, with a business address at 840 Memorial Drive Cambridge, Massachusetts (successor in interest as stated below, herein the "Lessee"), with respect to a certain Lease dated February 18, 2000 (as amended, as stated below) for certain office space in the building at 840 Memorial Drive Cambridge, Massachusetts.

WHEREAS, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain lease agreement dated February 18, 2000 (the "Original Lease");

WHEREAS, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain Amended and Restated Lease Agreement dated May 18, 2007 (the "First Amended Lease Agreement");

WHEREAS, Rivertech Associates II, LLC (as successor in interest to Rivertech Associates, LLC, the original signatory as Lessor under the Original Lease and First Amended Lease Agreement), and Genetix Pharmaceuticals, Inc., entered into a certain Lease Extension and Modification Agreement dated November 24, 2009 (the "First Lease Extension Agreement");

WHEREAS, Rivertech Associates II, LLC and Bluebird Bio, Inc. (a Delaware corporation, formerly known as Genetix Pharmaceuticals, Inc.), entered into a certain Second Amended and Restated Lease Agreement dated October 19, 2010 (the "Second Amended Lease Agreement");

WHEREAS, Rivertech Associates II, LLC and Bluebird Bio, Inc. entered into a certain Second Lease Extension and Modification Agreement" dated September 12, 2012 (the "Second Lease Extension Agreement");

WHEREAS, the Original Lease, as amended and modified by the foregoing First Amended Lease Agreement; Lease Extension Agreement; Second Amended Lease Agreement; and Second Lease Extension Agreement are collectively now referred to herein as the "Existing Lease" (updating redefining that term as it was used in the Second Lease Extension Agreement); and,

WHEREAS, by this Third Amended Lease Agreement Lessee seeks to lease, use and occupy an additional approximately 5,292 rentable square feet of office space located on the 4<sup>th</sup> floor of the Building, as shown on the Space Plan attached hereto as Exhibit A;

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**THEREFORE**, in consideration of One (\$1.00) Dollar and the other good and valuable consideration recited herein, effective and irrevocable as of the date hereof, the Lessor and Lessee hereby agree as follows:

**1. At Will Space Term / At Will Space Termination**

The At Will Space is the area on the fourth (4<sup>th</sup>) floor of the Building, as shown on the "Space Plan" attached hereto as Exhibit A.

Lessee hereby agrees to lease the At Will Space from the Lessor for successive monthly periods commencing on August 1, 2013 and continuing for each successive calendar month thereafter, until Lessee's lease, use and occupancy of the At Will Space is terminated by a written notice delivered by either the Lessor or the Lessee to the other, at least thirty (30) days prior to the intended date for termination (the "At Will Space Termination Date") as stated therein, which intended date of termination shall be the last day of a calendar month (the "At Will Space Termination Notice").

Upon termination of the Lessee's lease, use and occupancy of the At Will Space on the At Will Space Termination Date by delivery of the At Will Space Termination Notice, the Lessee shall completely surrender and vacate the At Will Space on or before the At Will Space Termination Date, relinquishing all claims to use and occupancy thereof.

Notwithstanding the termination of the Lessee's use and occupancy of the At Will Space as aforesaid, the Lessee shall nevertheless remain fully bound under the terms and conditions of the Existing Lease, and shall continue to lease, use and occupy the Existing Space through the end of the Term of the Existing Lease.

**2. Terms and Conditions**

Lessee shall lease the At Will Space on the same terms and conditions of the Existing Lease, as modified by this Third Lease Amendment, with exception only for those provisions as to which Lessor and Lessee have already performed their obligations as of the date hereof, (for example, Lessor has heretofore delivered the Existing Premises and Lessee has accepted the same).

**3. Delivery and Condition of At Will Space**

Lessor shall deliver the At Will Space to the Lessee upon execution of this Third Lease Amendment.

The At Will Space is leased in an "AS/IS" condition as of the execution of this Third Lease Amendment, and Lessee acknowledges that it has viewed and investigated the At Will Space and confirms that it is suitable in its current condition in all respects for Lessee's purposes, accepts the same on that basis. Lessor is under no obligation to make any repairs, improvements or modifications to the At Will Space, in any manner. Notwithstanding the foregoing, Lessor will provide adequate HVAC and electrical service for the licensed space appropriate for typical office use in the Building.

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The Lessee shall be solely responsible, at its sole cost and expense, to add any improvements, fixtures or furnishings to the At Will Space as it desires for its use and occupancy (“Lessee’s Work”); provided the foregoing do not involve or impact any electrical, mechanical, plumbing HVAC, life safety, or structural elements servicing or within the At Will Space, and further provided all plans and specifications for any such Lessee’s Work are approved in advance in writing by Lessor, and Lessee expressly agrees to remove the same immediately upon termination of its lease, use and occupancy of the At Will Space and to restore it to its condition as upon delivery by Lessor. Lessee’s Work (as approved by Lessor) and all subsequent Lessee alterations to the Leased Premises that are performed by Lessee on or affecting the fire, life safety and/or sprinkler systems of the building shall be made in such a manner and under such conditions as to pose no adverse impact or interruption to such fire, life safety, and sprinkler systems, and so as not to delay, impair, or jeopardize the legal occupancy of other tenants in the Building as determined by Lessor and municipal fire and building inspection officials.

#### **4. Annual Base Rent and Additional Rent for the At Will Space**

In addition to all Annual Base Rent and Additional Rent payable by Lessee under the Existing Lease as of this Third Amended Lease Agreement, Lessee shall pay to Lessor Annual Base Rent and Additional Rent attributable to the At Will Space, as follows:

A. Annual Base Rent Attributable to the At Will Space

Lessee shall pay to Lessor Annual Base Rent attributable to the At Will Space in the amount Twenty Two Thousand (\$ 22,000.00) Dollars per calendar month, for each month from August 1, 2013, until the At Will Space Termination Date.

In all instances, Annual Base Rent shall be due on the first of each month, in advance, and in all other respects shall be subject to the provisions relating to Annual Base Rent as set forth under the Existing Lease to the extent applicable.

B. Additional Rent Attributable to the At Will Space

Lessee shall pay to Lessor Additional Rent Attributable to the At Will Space only as follows (notwithstanding any other provisions of the Existing Lease calling for payment of Additional Rent under the Existing Lease), from August 1, 2013 until the At Will Space Termination Date:

- (a) all electrical use for the At Will Space, which will be separately metered to the At Will Space, based on invoices presented by the Lessor, and payable as directed by the Lessor (i.e. directly to the electric company or reimbursed to the Lessor), at the intervals invoiced by Lessor.

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C. At Will Space Annual Base Rent and At Will Space Additional Rent Supplement Payments due under the Existing Lease – General Provisions Apply

The payments specified under paragraphs 4A and 4B above supplement the payments of Annual Base Rent and Additional Rent required under the Existing Lease

All At Will Space Annual Base Rent and At Will Space Additional Rent shall be governed by the terms and conditions contemplated under the Existing Lease with respect to Annual Base Rent and Additional Rent, except to the extent modified and supplemented in paragraphs 4A and 4B above.

D. Security Deposit

The Security Deposit currently held by the Lessor shall apply as well to the obligations of the Lessee under this Third Amended Lease Agreement.

**5. Permitted Uses**

The Permitted Uses for the At Will Space shall be general office use, only. Lessee shall comply with all federal, state, municipal and local laws, codes, ordinances, rules and regulations of governmental authorities, committees, associations, or other regulatory committees, agencies or governing bodies having jurisdiction, and with the conditions and restrictions on use set forth in the Existing Lease (which are hereby restated and affirmed by Lessee).

**6. Existing Premises in “AS/IS” Condition – No Defaults**

Lessee accepts the At Will Space in its current “AS/IS” condition in all respects, without representation or warranty of any kind or nature by Lessor.

Lessor and Lessee each acknowledge that to the best of each of their respective knowledge, there are no material defaults by either presently existing under the Lease.

**7. Brokers**

The parties hereby agree there are no brokerage or other third party fees or costs involved in this transaction and each agrees to indemnify, defend and hold harmless the other from and against any claims for brokerage fees, commissions or other such payments arising from this transaction.

**8. Access; Elevators; Dumpsters; Security**

Lessee shall have 24/7 access to the At Will Space, the common areas and elevators serving the At Will Space, the freight elevators servicing the Building, the loading docks servicing the Building and the dumpster and/or compactor servicing the Building.

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Lessee shall be solely responsible for the security system(s) for the At Will Space, and shall provide Lessor with a description of the security measures to be taken pursuant thereto. Lessee waives and releases Lessor from and against any and all liability for any harm, damage or claims to persons or property in the At Will Space, and agrees to indemnify, hold harmless and defend Lessor against any and all such claims, except to the extent arising from Lessor's gross negligence or willful misconduct.

**9. Lessor's Rights Reserved**

Lessor reserves the right, upon reasonable advance notice to Lessee, to show the At Will Space to prospective tenants, current and prospective mortgagees, and other third parties during business hours. Lessor shall not materially interfere in Lessee's use and occupancy of the At Will Space during such showings.

Lessor reserves the right to request, and Lessee agrees to execute, the Lessor's lender's standard Subordination Non-Disturbance and Attornment Agreement; but Lessor shall have no obligation to procure the same.

**10. Integration of Documents; Supremacy**

This Third Amended Lease Agreement contains the full understanding and agreement between the parties. The parties hereto intend that this Third Amended Lease Agreement operates to amend and modify the Existing Lease, and that those documents shall be interpreted conjunctively; with any express conflict between the two to be resolved in favor of the stated terms of this Third Amended Lease Agreement. Except as expressly modified hereby, all other terms and conditions of the Existing Lease shall remain unchanged and enforceable in a manner consistent with this Third Amended Lease Agreement. Defined terms used in this Third Amended Lease Agreement that are not otherwise defined herein shall have the meaning ascribed to such terms in the Existing Lease.

This Third Amended Lease Agreement shall be governed by the laws of the Commonwealth of Massachusetts. Any provisions deemed unenforceable shall be severable, and the remainder of this Third Amended Lease Agreement and the Existing Lease shall be enforceable in accordance with their terms.

**[Signature Pages Follow]**

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Witness our hands and seals as of the date first written above.

**LESSOR**

**RIVERTech ASSOCIATES II, LLC**

By: /s/ Robert Epstein  
its duly authorized Manager

**LESSEE**

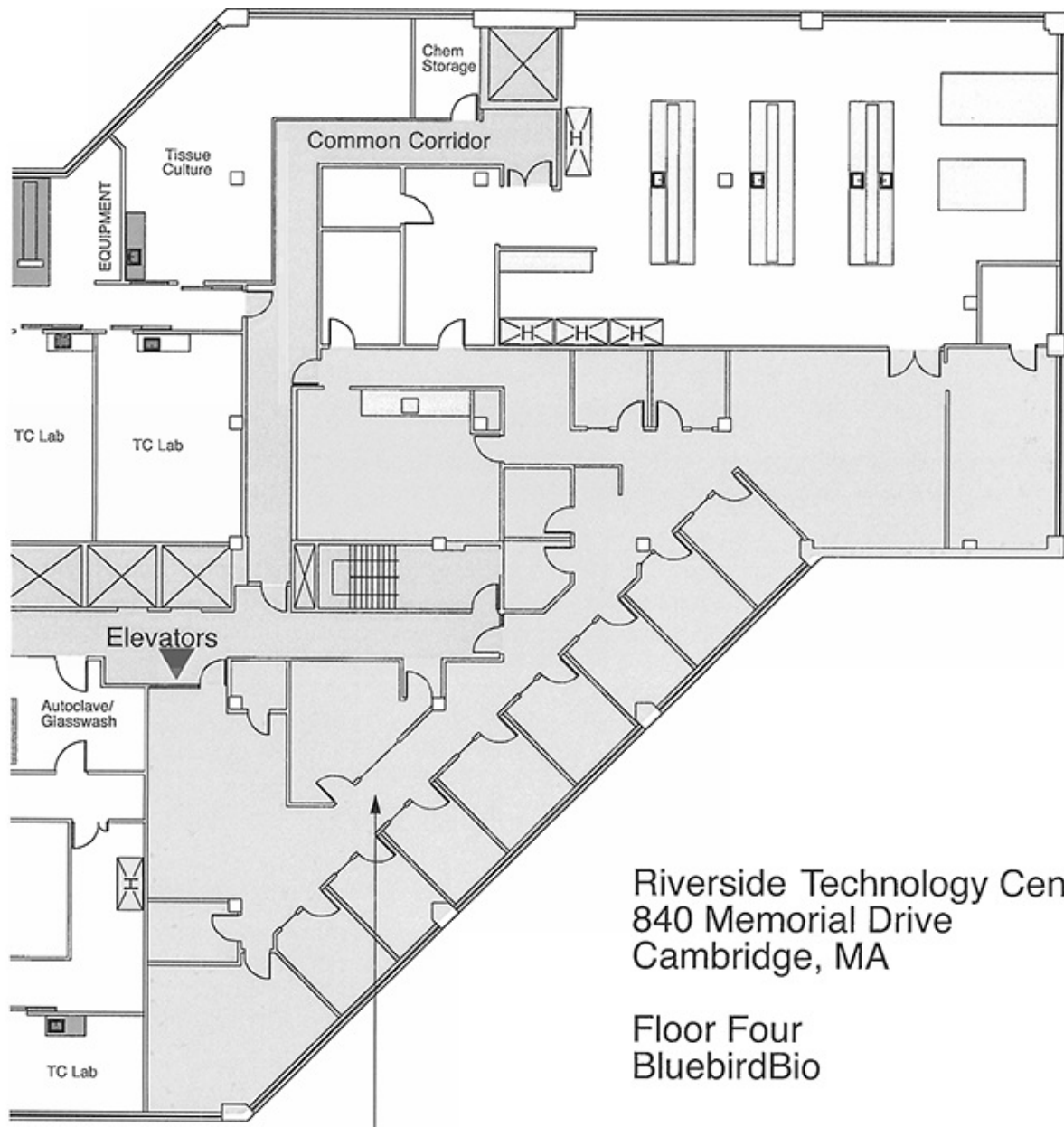
**BLUEBIRD BIO, INC.**

By: /s/ Jeffrey T. Walsh  
its duly authorized President/Vice President

By: /s/ Linda C. Bain  
Its duly authorized Treasurer/Ass't Treasurer



**See Space Plan for At Will Space Attached**



Riverside Technology Center  
 840 Memorial Drive  
 Cambridge, MA

Floor Four  
 BluebirdBio

At Will Space  
 5,292RSF

- Remaining Premises
- At Will Space

CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT WERE OMITTED AND REPLACED WITH “[\*\*\*]”. A COMPLETE VERSION OF THIS EXHIBIT HAS BEEN FILED SEPARATELY WITH THE SECRETARY OF THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO AN APPLICATION REQUESTING CONFIDENTIAL TREATMENT PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

### AMENDMENT N°3 TO THE LICENSE AGREEMENT

Between

**INSTITUT PASTEUR**, a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897, represented by M. Christophe Mauriet, Senior Executive Vice-President for Administration

Hereinafter referred to as “Institut Pasteur”

On one hand,

And

**BLUEBIRDBIO, INC.**, a company incorporated under the laws of Delaware, with offices at 840 Memorial Drive, Cambridge, MA 02139, United States, represented by Nick Leschly, Chief Executive Officer

Hereinafter referred to as “Licensee”,

On the other hand,

Hereinafter mentioned as a Party or the Parties.

#### Recitals

1. The Parties have signed a license agreement on September 13, 2011 on a patented specific nucleotide sequence having a triplex structure, referred to as “DNA flap”, modified by an amendment n°1 dated April 27, 2012 and amendment n°2 dated October 16, 2012 (the “Agreement”).
2. The Licensee has initiated a program to treat cancerous and/or pre-cancerous conditions by genetically modifying T cells. Given the potential utility of these same products and other modified T-cell products to treat and prevent [\*\*\*], the Parties now agree to include [\*\*\*] within the licensed Field.
3. The Parties now agree to further expand the Field to include human and non-human lentivirus.
4. The Parties agree to clarify the sublicensing terms of the Agreement.

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CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT WERE OMITTED AND REPLACED WITH “[\*\*\*]”. A COMPLETE VERSION OF THIS EXHIBIT HAS BEEN FILED SEPARATELY WITH THE SECRETARY OF THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO AN APPLICATION REQUESTING CONFIDENTIAL TREATMENT PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

**Now, therefore, the Parties hereby agree as follow:**

**Article 1. Scope**

The scope of this Amendment n°3 is to expand the Field of the Agreement and the license granted by Institut Pasteur, and to clarify the sublicensing terms.

**Article 2. Modifications**

2.1. The following definition in the Agreement is hereby amended and restated as follows:

- “Field” shall mean ex vivo Gene therapy for human disorders limited to adrenoleukodystrophy (ALD) (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta hemoglobinopathies (including but not limited to beta-thalassemia and sickle cell anemia), [\*\*\*] leukemias, lymphomas, B-cell malignancies and solid tumors by producing chimeric antigen receptor T-cells [\*\*\*]; for [\*\*\*] and [\*\*\*] the “Field” includes in vivo as well as ex vivo Gene therapy. [\*\*\*]
  - i For clarity, the Field excludes: (i) any other fields and specifically [\*\*\*] and (ii) the making, using, selling or offering for sale, and importation of [\*\*\*], and provision of services relating to the same.
  - j Institut Pasteur reserves the right to revoke the extension of the Field in article 2.1 of Amendment n°3 with respect to [\*\*\*] (but no other part of the Field) if and only if all of the following requirements are satisfied:
    - (a) at least [\*\*\*] months have elapsed since the date of the last signature by the Parties of Amendment n°3, and
    - (b) Licensee, its Affiliates, Licensee’s sublicensees or Third Party collaborators have not yet initiated a research program in the field of [\*\*\*] at the time of Institut Pasteur’s written inquiry but in any case within [\*\*\*] days of such inquiry.

For clarity, if Institut Pasteur believes that all of the foregoing requirements have been satisfied and wishes to exercise its rights to revoke the extension of the Field in article 2.1 of Amendment n°3 with respect to [\*\*\*] as provided above, Institut Pasteur must first confirm in writing to Licensee the revocation of the [\*\*\*] Field.

2.2 Article 2.1 of the Agreement is hereby amended and restated as follows:

- “2.1. Under this Agreement, Institut Pasteur hereby grants to Licensee and its Affiliates, that Licensee and its Affiliates accept at their own risk, a license under the Patents in the Field and in the Territory for research and development, and to manufacture, have manufactured, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import or have imported the Products, to the exclusion of any other rights, which is:
  - exclusive for Products containing or based on any lentivirus vector (including human and non-human lentivirus).

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CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT WERE OMITTED AND REPLACED WITH “[\*\*\*]”. A COMPLETE VERSION OF THIS EXHIBIT HAS BEEN FILED SEPARATELY WITH THE SECRETARY OF THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO AN APPLICATION REQUESTING CONFIDENTIAL TREATMENT PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

In addition, Institut Pasteur hereby grants to Licensee, and its Affiliates, that Licensee and its Affiliates accept at their own risk, a nonexclusive license under the Patents in the Field and in the Territory to make or to have made by a Third Party Good Manufacturing Practice (GMP) batches of lentiviral vectors for its/their own clinical trials on Products, provided that such Third Party makes Good Manufacturing Practice (GMP) batches of lentiviral vectors solely for the Licensee and its Affiliates and sublicensees clinical trials of Products above mentioned.

2.3 Article 2.3 of the Agreement is hereby amended and restated as follows:

“This Agreement includes the right for Licensee and/or Affiliates to grant sublicenses in the Field and the Territory to and through multiple tier(s) of Third Party(ies) provided that Licensee complies with the following conditions:

- Each sublicense is consistent with the terms of the present Agreement and is no less protective of Institut Pasteur than the terms of this Agreement,
- Licensee advises Institut Pasteur at the earliest reasonable possible moment if Licensee intends and expects to grant a sublicense, including the material proposed terms thereof,
- Licensee provides Institut Pasteur with a copy of each signed sublicense agreement to Institut Pasteur within thirty (30) days after execution thereof (subject to required redaction of sensitive information, provided that all financial terms relating to Technology and/or Products are disclosed to Institut Pasteur ),
- Licensee and/or its Affiliates shall be responsible for its sublicensees, and for diligently collecting all amounts due to Licensee from sublicensees,
- If a sublicensee pursuant hereto becomes bankrupt, insolvent or is placed in the hands of a receiver or trustee, Licensee, to the extent allowed under applicable law and in a timely manner, agrees to use its best reasonable efforts to collect all consideration owed to Licensee.

2.4. Article 6.7 of the Agreement is hereby clarified as follows:

“6.7. For sublicenses relating to a Product other than relating to ALD (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta-thalassemia and/or sickle cell anemia, the following shall apply:

- On an indication-by-indication basis, Licensee and/or Affiliates shall pay to Institut Pasteur on any and all cash and cash-equivalent consideration, whatever its nature, and in particular without limitation, all sums, milestones, royalties, exchange value of any counterpart in kind or in industry (but not duly justified payments for research and development) received by Licensee and/or Affiliates from a sublicensee:
  - [\*\*\*] if the sublicense is signed for such Product(s) in a preclinical stage development, or,
  - [\*\*\*] if the sublicense is signed for such Product(s) in a clinical stage of development.
- In the event that Licensee, and/or its Affiliates, is required to pay amounts to Institut Pasteur pursuant to this article 6.7 in consideration of a sublicense, for such Product(s) then Licensee and/or Affiliates shall not be required to pay any other consideration to Institut Pasteur arising from any such sublicenses, including but not limited to under articles 6.2, 6.3, 6.5, and 6.6.

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CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT WERE OMITTED AND REPLACED WITH “[\*\*\*]”. A COMPLETE VERSION OF THIS EXHIBIT HAS BEEN FILED SEPARATELY WITH THE SECRETARY OF THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO AN APPLICATION REQUESTING CONFIDENTIAL TREATMENT PURSUANT TO RULE 24b-2 PROMULGATED UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED.

For clarity, article 6.9 shall apply in case of any sublicenses relating to a Product for ALD (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta-thalassemia and/or sickle cell anemia.”

**Article 3. Other Terms**

3.1. Upon execution of this Amendment n°3 by the Parties, Licensee will pay Institut Pasteur [\*\*\*].

**Article 4. Miscellaneous**

4.1. All the other provisions of the Agreement remain unchanged and fully applicable between the Parties.

4.2. This Amendment n°3 is effective from the date of signature by the Parties.

4.3. This Amendment n°3 shall be an integral part of the Agreement.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their authorized respective representative.

Made in Paris,  
In duplicate.

Date: 10 SEPT. 2013

/s/ Christophe Mauriet

\_\_\_\_\_  
INSTITUT PASTEUR  
Christophe Mauriet  
Senior Executive Vice-President  
for Administration

Date: 10 SEPT. 2013

/s/ Nick Leschly

\_\_\_\_\_  
BLUEBIRDBIO, INC.  
Nick Leschly  
Chief Executive Officer

## CERTIFICATIONS

I, Nick Leschly, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of bluebird bio, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 14, 2013

By: /s/ Nick Leschly

Nick Leschly  
President and Chief Executive Officer  
(Principal Executive Officer)

## CERTIFICATIONS

I, Jeffrey T. Walsh, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of bluebird bio, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 14, 2013

By: /s/ Jeffrey T. Walsh

Jeffrey T. Walsh  
Chief Operating Officer and Secretary  
(Principal Financial Officer)



**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of bluebird bio, Inc. (the "Company") for the period ended September 30, 2013 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his or her knowledge:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 14, 2013

By: /s/ Nick Leschly

Nick Leschly  
President and Chief Executive Officer  
(Principal Executive Officer)

Date: November 14, 2013

By: /s/ Jeffrey T. Walsh

Jeffrey T. Walsh  
Chief Operating Officer and Secretary  
(Principal Financial Officer)

