bluebird bio, Inc.
Form 10-Q
November 02, 2016

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended September 30, 2016

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

bluebird bio, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	13-3680878
(State or Other Jurisdiction of Incorporation or Organization)	(IRS Employer Identification No.)
150 Second Street	02141

Cambridge, Massachusetts
(Address of Principal Executive Offices) (Zip Code)

(339) 499-9300

(Registrant's Telephone Number, Including Area Code)

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 28, 2016, there were 37,303,678 shares of the registrant's Common Stock, par value $0.01 per share, outstanding.

This Quarterly Report on Form 10-Q contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. 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," "target," "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 and drug product manufacturing capabilities;
- the timing or likelihood of regulatory filings and approvals for our product candidates;
- the timing or success of 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 and licenses;
- 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.

bluebird bio, Inc.

Form 10-Q

For the three and nine months ended September 30, 2016

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

bluebird bio, Inc.

Condensed Consolidated Balance Sheets

(unaudited)

(in thousands, except par value amounts)

	September 30, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 245,154	$ 164,269
Marketable securities	355,806	353,680
Prepaid expenses and other current assets	7,875	6,016
Total current assets	608,835	523,965
Marketable securities	126,681	347,814
Property and equipment, net	131,737	82,614
Intangible assets, net	21,634	24,456
Goodwill	13,128	13,128
Restricted cash and other non-current assets	16,247	10,360
Total assets	$ 918,262	$ 1,002,337
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 6,710	$ 6,334
Accrued expenses and other current liabilities	54,672	28,145
Deferred revenue, current portion	6,209	5,889
Total current liabilities	67,591	40,368
Deferred rent, net of current portion	10,995	8,294
Deferred revenue, net of current portion	41,756	35,959
Contingent consideration, net of current portion	3,267	5,082
Construction financing lease obligation	99,991	61,901
Other non-current liabilities	149	237
Total liabilities	223,749	151,841
Commitments and contingencies (Note 7)		
Stockholders' equity:		
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at September 30, 2016 and December 31, 2015	—	—
Common stock, $0.01 par value, 125,000 shares authorized; 37,296 and 36,894 shares	373	369

issued and outstanding at September 30, 2016 and December 31, 2015, respectively		
Additional paid-in capital	1,201,286	1,166,585
Accumulated other comprehensive loss	(836)	(2,291)
Accumulated deficit	(506,310)	(314,167)
Total stockholders' equity	694,513	850,496
Total liabilities and stockholders' equity	$ 918,262	$ 1,002,337

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 September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Revenue:				
Collaboration revenue	$1,552	$1,324	$4,603	$12,607
Total revenue	1,552	1,324	4,603	12,607
Operating expenses:				
Research and development	63,971	30,395	147,642	98,380
General and administrative	14,623	13,704	48,941	31,765
Change in fair value of contingent consideration	1,098	352	3,515	2,540
Total operating expenses	79,692	44,451	200,098	132,685
Loss from operations	(78,140)	(43,127)	(195,495)	(120,078)
Other income, net	937	263	2,803	630
Loss before income taxes	(77,203)	(42,864)	(192,692)	(119,448)
Income tax benefit (expense)	178	(60)	549	(60)
Net loss	$(77,025)	$(42,924)	$(192,143)	$(119,508)
Net loss per share - basic and diluted:	$(2.07)	$(1.18)	$(5.19)	$(3.52)
Weighted-average number of common shares used in computing net loss per share - basic and diluted:	37,201	36,384	37,026	33,979
Other comprehensive (loss) income:				
Unrealized (loss) gain on available-for-sale securities, net of tax (benefit) expense of ($0.1) and $0.8 million for the three and nine months ended September 30, 2016, respectively	(194)	103	1,455	121
Comprehensive loss	$(77,219)	$(42,821)	$(190,688)	$(119,387)

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Condensed Consolidated Statements of Cash Flows

(unaudited)

(in thousands)

	Nine months ended September 30,	
	2016	2015
Operating activities		
Net loss	$(192,143)	$(119,508)
Adjustments to reconcile net loss to net cash used in operating activities:		
Change in fair value of contingent consideration	2,099	2,015
Depreciation and amortization	7,132	5,381
Stock-based compensation expense	30,831	31,011
Other non-cash items	2,166	528
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(3,857)	(2,717)
Accounts payable	1,210	623
Accrued expenses and other liabilities	24,099	5,320
Deferred revenue	6,117	12,643
Deferred rent	1,805	(640)
Net cash (used in) operating activities	(120,541)	(65,344)
Investing activities		
Restricted cash	(4,379)	(8,816)
Purchase of property and equipment	(15,005)	(3,618)
Purchases of marketable securities	(145,135)	(470,499)
Proceeds from maturities of marketable securities	356,684	132,239
Proceeds from sales of marketable securities	7,500	—
Net cash provided by (used in) investing activities	199,665	(350,694)
Financing activities		
Cash paid for contingent purchase price consideration	(2,025)	(453)
Proceeds from public offering of common stock, net of issuance costs	—	477,247
Proceeds from issuance of common stock	3,786	8,909
Net cash provided by financing activities	1,761	485,703
Increase in cash and cash equivalents	80,885	69,665
Cash and cash equivalents at beginning of period	164,269	347,845
Cash and cash equivalents at end of period	$245,154	$417,510
Non-cash investing and financing activities:		
Construction financing lease obligation	$38,090	$43,777
Purchases of property and equipment included in accounts payable and accrued expenses	$2,479	$1,475
Stock option exercise proceeds receivable	$374	$24

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

1. Description of the business

bluebird bio, Inc. (the “Company” or “bluebird”) was incorporated in Delaware on April 16, 1992, and is headquartered in Cambridge, Massachusetts. The Company researches, develops, manufactures and plans to commercialize gene therapies for the treatment of severe genetic and rare diseases and in the field of T cell-based immunotherapy. Since its inception, the Company has devoted substantially all of its resources to its research and development efforts relating to its product candidates, including activities to manufacture product candidates, conduct clinical studies of its product candidates, perform preclinical research to identify new product candidates and provide general and administrative support for these operations.

2. Summary of significant accounting policies and basis of presentation

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, 2016 and 2015.

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, 2015, and the notes thereto, which are included in the Company’s Annual Report on Form 10-K filed with the Securities and Exchange Commission (the “SEC”) on February 25, 2016.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries: Precision Genome Engineering, Inc. (“Pregenen”), bluebird bio France – SARL, bluebird bio Australia Pty Ltd., bluebird bio (UK) Ltd., bluebird bio (Bermuda) Ltd. 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.

Summary of accounting policies

The significant accounting policies and estimates used in the preparation of the condensed consolidated financial statements are described in the Company's audited financial statements as of and for the year ended December 31, 2015, and the notes thereto, which are included in the Company's Annual Report on Form 10-K. There have been no material changes in the Company's significant accounting policies during the nine months ended September 30, 2016.

Net income (loss) per share

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 stock equivalent shares outstanding for the period, including any dilutive effect from outstanding stock options, unvested restricted stock, restricted stock units, employee stock purchase plan, and warrants using the treasury stock method.

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. Estimates are used in the following areas, among others: fair value estimates used to assess potential impairment of long-lived assets, construction financing lease obligations, contingent consideration, stock-based compensation expense, accrued expenses, revenue and income taxes. Actual results could materially differ from those estimates.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

Recently issued accounting pronouncements

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (“Topic 606”), which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. The new standard will be effective on January 1, 2018 and earlier application is permitted only for annual reporting periods beginning after December 15, 2016, including interim reporting periods within that reporting period. The Company is currently evaluating the potential impact that Topic 606 may have on its financial position and results of operations.

In February 2016, the FASB issued ASU 2016-02, Leases, (“ASU 2016-02”), which requires a lessee to recognize assets and liabilities on the balance sheet for operating leases and changes many key definitions, including the definition of a lease. The new standard includes a short-term lease exception for leases with a term of 12 months or less, as part of which a lessee can make an accounting policy election not to recognize lease assets and lease liabilities. Lessees will continue to differentiate between finance leases (previously referred to as capital leases) and operating leases using classification criteria that are substantially similar to the previous guidance. The new standard will be effective beginning January 1, 2019, and early adoption is permitted for public entities. The Company is currently evaluating the potential impact ASU 2016-02 may have on its financial position and results of operations.

In March 2016, the FASB issued ASU 2016-09, Improvements to Employee Share-Based Payment Accounting (“ASU No. 2016-09”), which simplifies share-based payment accounting through a variety of amendments. The standard will be effective for annual reporting periods and interim periods within those annual periods, beginning after December 15, 2016, and early adoption is permitted. The Company is currently evaluating the potential impact ASU 2016-09 may have on its financial position and results of operations.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments (“Topic 230”). The new standard clarifies certain aspects of the statement of cash flows, including the classification of contingent consideration payments made after a business combination and several other clarifications not currently applicable to the Company. The new standard also clarifies that an entity should determine each separately identifiable source or use within the cash receipts and cash payments on the basis of the nature of the underlying cash flows. In situations in which cash receipts and payments have aspects of more than one class of cash flows and cannot be separated by source or use, the appropriate classification should depend on the activity that is likely to be the predominant source or use of cash flows for the item. The new standard will be effective for the Company on January 1, 2018. The adoption of this standard is not expected to have a material impact on our statements of cash flows upon adoption.

3. Marketable securities

The following table summarizes the available-for-sale securities held at September 30, 2016 and December 31, 2015 (in thousands):

Description	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
September 30, 2016				
U.S. government agency securities and treasuries	$475,344	$ 91	$ (164)	$475,271
Certificates of deposit	7,200	16	—	7,216
Total	$482,544	$ 107	$ (164)	$482,487
December 31, 2015				
U.S. government agency securities and treasuries	$689,425	$ 22	$ (2,300)	$687,147
Certificates of deposit	14,360	—	(13)	14,347
Total	$703,785	$ 22	$ (2,313)	$701,494

No available-for-sale securities held as of September 30, 2016 or December 31, 2015 had remaining maturities greater than three years.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

4. Fair value measurements

The following table sets forth the Company's assets and liabilities that are measured at fair value on a recurring basis as of September 30, 2016 and December 31, 2015 (in thousands):

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
September 30, 2016				
Assets:				
Cash and cash equivalents	$245,154	$245,154	$—	$ —
Marketable securities:				
U.S. government agency securities and treasuries	475,271	—	475,271	—
Certificates of deposit	7,216	—	7,216	—
Total assets	$727,641	$245,154	$482,487	$ —
Liabilities:				
Contingent consideration	$7,180	$—	$—	$ 7,180
Total liabilities	$7,180	$—	$—	$ 7,180
December 31, 2015				
Assets:				
Cash and cash equivalents	$164,269	$158,269	$6,000	$ —
Marketable securities:				
U.S. government agency securities and treasuries	687,147	—	687,147	—
Certificates of deposit	14,347	—	14,347	—
Total assets	$865,763	$158,269	$707,494	$ —
Liabilities:				
Contingent consideration	$8,665	$—	$—	$ 8,665
Total liabilities	$8,665	$—	$—	$ 8,665

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, 2016, cash and cash equivalents comprise funds in cash, money market accounts, and federally insured deposits. As of December 31, 2015, cash and cash equivalents comprise

funds in cash, money market accounts, U.S. Treasury securities and federally insured deposits.

Marketable securities

The amortized cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity. At September 30, 2016 and December 31, 2015, the balance in the Company's accumulated other comprehensive loss was composed solely of activity related to the Company's available-for-sale marketable securities. There were no material realized gains on the sale or maturity of available-for-sale securities during the nine months ended September 30, 2016, and as a result, the Company did not reclassify any material amounts out of accumulated other comprehensive loss for the same period.

The aggregate fair value of securities held by the Company in an unrealized loss position for less than twelve months as of September 30, 2016 and December 31, 2015 was $255.8 million and $638.1 million, respectively. There were no securities held by the Company in an unrealized loss position for more than twelve months as of September 30, 2016. The Company has the intent and ability to hold such securities until recovery. The Company determined that there was no material change in the credit risk of the above investments. As a result, the Company determined it did not hold any investments with any other-than-temporary impairment as of September 30, 2016 and December 31, 2015.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

Contingent consideration

On June 30, 2014, the Company acquired Pregenen. In connection with the acquisition, the Company recorded contingent consideration pertaining to the amounts potentially payable to Pregenen's former equityholders pursuant to the Stock Purchase Agreement (the "Stock Purchase Agreement") by and among the Company, Pregenen and Pregenen's former equityholders. Contingent consideration is measured at fair value and is based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The valuation of contingent consideration uses assumptions the Company believes would be made by a market participant. The Company assesses these estimates on an on-going basis as additional data impacting the assumptions is obtained. Future changes in the fair value of contingent consideration related to updated assumptions and estimates are recognized within the condensed consolidated statements of operations and comprehensive loss.

Contingent consideration may change significantly as development progresses and additional data are obtained, impacting the Company's assumptions regarding probabilities of successful achievement of related milestones used to estimate the fair value of the liability and the timing in which they are expected to be achieved. In evaluating the fair value information, considerable judgment is required to interpret the market and internal data used to develop the estimates. The estimates of fair value may not be indicative of the amounts that could be realized in a current market exchange. Accordingly, the use of different market and internal assumptions and/or different valuation techniques could result in materially different fair value estimates.

The significant unobservable inputs used in the measurement of fair value of the Company's contingent consideration are probabilities of successful achievement of preclinical, clinical and commercial milestones, the period in which these milestones are expected to be achieved ranging from 2017 to 2026 and discount rates ranging from 9.9% to 12.7%. Significant increases or decreases in any of the probabilities of success would result in a significantly higher or lower fair value measurement, respectively. Significant increases or decreases in these other inputs would result in a significantly lower or higher fair value measurement, respectively.

The table below provides a roll-forward of fair value of the Company's contingent consideration obligations, which include Level 3 inputs (in thousands):

	Nine Months Ended September 30, 2016
Beginning balance	$ 8,665
Additions	—
Changes in fair value	3,515
Payments	(5,000)
Ending balance	$ 7,180

The Company may be required to make up to $129.0 million in remaining future contingent cash payments to the former equityholders of Pregenen upon the achievement of certain milestones related to the Pregenen technology, of which $9.0 million relates to preclinical milestones, $20.1 million relates to clinical milestones, and $99.9 million relates to commercial milestones. As of September 30, 2016, $3.9 million of the fair value of the Company's total contingent consideration obligations was reflected as a component of accrued expenses and other current liabilities within the condensed consolidated balance sheets, with the remaining balance of $3.3 million reflected as a non-current liability.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

5. Property and equipment, net

Property and equipment, net, consists of the following (in thousands):

	September 30, 2016	December 31, 2015
Computer equipment and software	$ 1,350	$ 1,259
Office equipment	1,427	1,104
Laboratory equipment	15,273	10,520
Leasehold improvements	13,839	11,010
Construction-in-progress	110,922	65,542
Total property and equipment, gross	142,811	89,435
Less accumulated depreciation and amortization	(11,074)	(6,821)
Total property and equipment, net	$ 131,737	$ 82,614

Construction-in-progress as of September 30, 2016 includes $109.7 million related to construction costs at 60 Binney Street in Cambridge, Massachusetts, of which $100.0 million was incurred by the landlord. Please refer to Note 7, "Commitments and contingencies," for further information.

6. Accrued expenses and other current liabilities

Accrued expenses and other current liabilities consist of the following (in thousands):

	September 30, 2016	December 31, 2015
Employee compensation	$ 8,920	$ 5,935
Accrued goods and services	23,855	15,876
Accrued license and milestone fees	15,208	277
Accrued professional fees	2,103	1,014
Deferred rent, current portion	68	964
Contingent consideration, current portion	3,913	3,584
Other	605	495
Total accrued expenses and other current liabilities	$ 54,672	$ 28,145

Accrued license and milestone fees as of September 30, 2016 includes a $15.0 million upfront fee related to a research collaboration and license agreement entered into during the third quarter of 2016. This upfront fee was paid in the fourth quarter of 2016.

7. Commitments and contingencies

The Company is party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at September 30, 2016 and December 31, 2015 or royalties on future sales of specified products.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to these 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 claims by any third party with respect to the Company's products or business activities. 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.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

The Company's wholly-owned subsidiary bluebird bio France – SARL participates in the French Crédit d'Impôt Recherche ("CIR") program, which allows companies to monetize up to 30% of eligible research expenses. As of September 30, 2016, the Company received aggregate reimbursement of €2.3 million related to years 2013 through 2015. The Company has not yet applied for the €0.6 million related to the nine months ended September 30, 2016. The years 2013 through 2016 are open and subject to examination.

Operating Lease Commitments

On June 3, 2013, the Company entered into a nine-year building lease for approximately 43,600 square feet of space located at 150 Second Street, Cambridge, Massachusetts, which commenced in December 2013. This lease was amended in June 2014 to add approximately 9,900 square feet. The lease originally had monthly lease payments of $0.2 million for the first 12 months, which increased to $0.3 million per month beginning in December 2014 due to the lease amendment, with annual rent escalations thereafter. Rent expense is recognized on a straight-line basis over the term of the lease. The Company has the option to extend this lease by an additional five years. The lease provided a contribution from the landlord towards the initial build-out of the space of up to $7.8 million. 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. In addition, in accordance with the lease, the Company entered into a cash-collateralized irrevocable standby letter of credit in the amount of $1.3 million, naming the landlord as beneficiary, which has a balance of $0.6 million as of September 30, 2016.

On September 30, 2016, the Company entered into an Assignment and Assumption of Lease ("Assignment") relating to its lease at 150 Second Street. Under the Assignment, the Company will assign all of its rights, interests, obligations and responsibilities under the lease, to be effective as of the later of May 1, 2017 or the first day following the Company's surrender of the leased premises in accordance with the lease. The Company expects to vacate the premises by mid-2017 and as a result, no longer expects to pay $20.6 million in lease payments between 2017 and 2022, including $2.2 million in 2017, $3.5 million in 2018, $3.6 million in 2019, $3.7 million in 2020 and $7.6 million in 2021 and thereafter.

On June 29, 2015, the Company entered into a lease agreement for additional office space located at 215 First Street, Cambridge, Massachusetts. Under the terms of the lease, the Company leased approximately 15,120 square feet starting on July 13, 2015 for $0.5 million per year in base rent, which is subject to a 3% annual rent increase plus certain operating expenses and taxes. The lease will continue until the end of the 60th full calendar month following the date the landlord delivers the premises to the Company, and includes early termination provisions that could allow the Company to terminate the lease without penalty at the end of the 20th full calendar month following the delivery of the premises if the Company meets certain conditions specified within the lease. Under the terms of the lease, the Company has also leased an additional 8,075 square feet of office space in the same premises starting on January 1, 2016 for an additional $0.3 million per year in base rent, which is subject to a 3% annual rent increase plus certain operating expenses and taxes. The Company expects to terminate the lease by mid-2017.

On June 3, 2016, the Company entered into a strategic manufacturing agreement for the future commercial production of the Company's Lenti-D and LentiGlobin product candidates with a contract manufacturing organization. Under this 12 year agreement, the contract manufacturing organization will complete the design, construction, validation and process validation of the leased suites prior to anticipated commercial launch of the product candidates. During construction, the Company is required to pay $12.5 million upon the achievement of certain construction milestones, and may pay up to $8.0 million in additional construction milestones if the Company elects its option to lease additional suites. The Company paid $2.0 million for the achievement of the first milestone during the third quarter of 2016, which is reflected as a component of other non-current assets within the condensed consolidated balance sheets. Following construction completion, the Company will pay $5.1 million per year in fixed suite fees as well as certain fixed labor, raw materials, testing and shipping costs for manufacturing services, and may pay additional suite fees if it elects its option to reserve or lease additional suites. The Company may terminate this agreement any time after July 1, 2016 upon payment of a one-time termination fee and up to 24 months of fixed suite and labor fees. The Company concluded that this agreement contains an embedded lease as the suites are designated for the Company's exclusive use during the term of the agreement. The Company concluded that it is not the deemed owner during construction nor is it a capital lease. As a result, the Company will account for the agreement as an operating lease and expense the rental payments on a straight-line basis over the term of the embedded lease.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

60 Binney Street Lease Commitments

On September 21, 2015, the Company entered into a lease agreement, which was amended for certain administrative matters on June 21, 2016, for additional office and laboratory space located in a building (the "Building") under construction at 60 Binney Street, Cambridge, Massachusetts (the "60 Binney Lease"). Under the terms of the 60 Binney Lease, starting on October 1, 2016, the Company will lease approximately 253,108 square feet of office and laboratory space at $72.50 per square foot per year, or $18.4 million per year in base rent, which is subject to scheduled annual rent increases of 1.75% plus certain operating expenses and taxes. The Company also executed a $9.2 million letter of credit upon signing the 60 Binney Lease, which was required to be collateralized with a bank account at a financial institution in accordance with the 60 Binney Lease agreement. This letter of credit was increased to $13.8 million during the third quarter of 2016 as required under the terms of the lease. Subject to the terms of the lease and certain reduction requirements specified therein, including market capitalization requirements, this amount may decrease back to $9.2 million over time. The 60 Binney Lease will continue until the end of the 120th full calendar month following April 2017 or the earlier the date the Company occupies the Building or other conditions specified in the 60 Binney Lease occur. Pursuant to a work letter entered into in connection with the 60 Binney Lease, the landlord will contribute an aggregate of $42.4 million toward the cost of construction and tenant improvements for the Building. The purpose of the 60 Binney Lease is to supplement and eventually replace the Company's current leased premises at 150 Second Street and 215 First Street in Cambridge, Massachusetts and the Company intends to move its corporate headquarters to 60 Binney Street in mid-2017. The Company has the option to extend the 60 Binney Lease for two successive five-year terms.

Because the Company is involved in the construction project, including having responsibility to pay for a portion of the costs of finish work and mechanical, electrical, and plumbing elements of the Building, the Company is deemed for accounting purposes to be the owner of the Building during the construction period. Accordingly, the Company has recorded project construction costs incurred by the landlord as an asset in "Property and equipment, net" and a related financing obligation in "Construction financing lease obligation" on the Company's condensed consolidated balance sheet.

The Company bifurcates its future lease payments pursuant to the 60 Binney Lease into (i) a portion that is allocated to the Building and (ii) a portion that is allocated to the land on which the Building is being constructed, which is recorded as rental expense. Although the Company estimates that the Company will not begin making lease payments pursuant to the 60 Binney Lease until April 2017, the portion of the lease obligation allocated to the land is treated for accounting purposes as an operating lease that commenced upon execution of the 60 Binney Lease in September 2015. During the three and nine months ended September 30, 2016, the Company recognized $0.5 and $1.4 million of non-cash rental expense attributable to the land.

As of September 30, 2016, Property and equipment, net, includes $109.7 million related to construction costs for the Building. The construction financing lease obligation related to the Building is $100.0 million. No cash has been paid to the landlord to date.

Once the landlord completes the construction of the Building, the Company will evaluate the 60 Binney Lease in order to determine whether or not the 60 Binney Lease meets the criteria for "sale-leaseback" treatment. If the 60 Binney Lease meets the "sale-leaseback" criteria, the Company will remove the asset and the related liability from its

consolidated balance sheet and treat the 60 Binney Lease as either an operating or a capital lease based on the Company's assessment of the accounting guidance. The Company expects that upon completion of construction of the Building the 60 Binney Lease will not meet the "sale-leaseback" criteria. If the 60 Binney Lease does not meet "sale-leaseback" criteria, the Company will treat the 60 Binney Lease as a financing obligation and will depreciate the asset in accordance with the Company's accounting policy.

8. Significant agreements

Celgene Corporation

Original Collaboration Agreement

On March 19, 2013, the Company entered into a Master Collaboration Agreement (the "Collaboration Agreement") with Celgene Corporation ("Celgene") to discover, develop and commercialize potentially 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 $75.0 million up-front, non-refundable cash payment. The Company was responsible for conducting discovery, research and development activities through completion of Phase I clinical studies, if any, during the initial term of the Collaboration Agreement, or three years. The collaboration is governed by a joint steering committee ("JSC") formed by an equal number of representatives from the Company and Celgene. The JSC, among other activities, reviews the collaboration program, reviews and evaluates product candidates and approves regulatory plans. In addition to the JSC, the Collaboration Agreement provides that the Company and Celgene each appoint representatives to a patent committee, which is responsible for managing the intellectual property developed and used during the collaboration.

Amended Collaboration Agreement

On June 3, 2015, the Company and Celgene amended and restated the Collaboration Agreement (the "Amended Collaboration Agreement"). Under the Amended Collaboration Agreement, the parties will now focus the collaboration exclusively on anti- B-cell maturation antigen ("BCMA") product candidates for a new three-year term. In connection with the Amended Collaboration Agreement, the Company received an upfront, one-time, non-refundable, non-creditable payment of $25.0 million to fund research and development under the collaboration. The collaboration will continue to be governed by the JSC.

Under the terms of the Amended Collaboration Agreement, for up to two product candidates selected for development under the collaboration, the Company is responsible for conducting and funding all research and development activities performed up through completion of the initial Phase I clinical study of such product candidate.

On a product candidate-by-product candidate basis, up through a specified period following enrollment of the first patient in an initial Phase I clinical study for such product candidate (the "Option Period"), the Company has granted Celgene an option to obtain an exclusive worldwide license to develop and commercialize such product candidate pursuant to a written agreement, the form of which the Company has already agreed upon. In the event that Celgene exercises its option with respect to any product candidate, the Company may elect to co-develop and co-promote the product candidate in the United States, provided that, if the Company does not exercise its option co-develop and co-promote the first product candidate in-licensed by Celgene under the Amended Collaboration Agreement, then the Company will not be permitted to exercise its option to co-develop and co-promote any future product candidates under the Amended Collaboration Agreement. If Celgene elects to exercise its option to exclusively in-license a product candidate, it must pay the Company an option fee in the amount of $10.0 million for the first product candidate and $15.0 million for any additional product candidates.

On February 10, 2016, Celgene exercised its option to obtain an exclusive worldwide license to develop and commercialize bb2121, the first product candidate under the Amended Collaboration Agreement, pursuant to an executed license agreement entered into by the parties on February 16, 2016 and paid the associated $10.0 million option fee. The Company may now elect to co-develop and co-promote the product candidate in the United States and will receive an additional fee in the amount of $10.0 million in the event the Company does not exercise its option to co-develop and co-promote bb2121 with Celgene. On February 17, 2016, the parties further amended the Amended Collaboration Agreement to update the timing of certain deliverables in connection with Celgene's option exercise for the license of the bb2121 product candidate.

Accounting Analysis

The Company's Amended Collaboration Agreement with Celgene contains the following deliverables: (i) research and development services, (ii) participation on the JSC, (iii) participation on the patent committee, (iv) a license to the first product candidate, (v) manufacture of vectors and associated payload for incorporation into the first optioned product candidate under the license, and (vi) participation on the JGC under the co-development and co-promotion agreement for the first optioned product candidate under the license.

The license to the first product candidate was considered a deliverable at the inception of the arrangement and therefore the associated option fee was included in allocable arrangement consideration as the Company believed there was minimal risk with regard to whether Celgene will exercise the option based on the successful completion of preclinical activities and proximity of enrollment of the first patient in an initial Phase I clinical study for this product candidate. The Company determined that the obligation within the license to manufacture or have manufactured supplies of vectors and associated payloads for incorporation into the first optioned product candidate is a deliverable, consistent with the option to license the first product candidate.

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However, the Company determined that the options to license any additional product candidates are substantive options and therefore were not considered deliverables at execution of the Amended Collaboration Agreement. 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 to license additional product candidates. Moreover, the Company determined that the options are not priced at a significant and incremental discount. Accordingly, the options to other product candidates are not considered deliverables and the associated option fees are not included in allocable arrangement consideration.

Upon execution of the Amended Collaboration Agreement in June 2015, the Company concluded that each of the three delivered elements at the inception of the agreement (research and development services, participation on the JSC and participation on the patent committee) had standalone value from the other undelivered elements. Additionally, the Amended Collaboration Agreement does not include return rights related to the collaboration term. Accordingly, each deliverable qualified as a separate unit of accounting.

The Company determined that each of the delivered elements had the same period of performance (the three year term through projected initial Phase I clinical study substantial completion) and the same pattern of revenue recognition, ratably over the period of performance as there was no other discernible pattern of recognition. The Company identified the allocable arrangement consideration as the $25.0 million up-front research and development funding payment, $10.0 million option fee for the first product candidate, $20.0 million related to remaining deferred revenue from the original Collaboration Agreement, and $54.1 million of contingent revenue related to the estimated amounts that will be received from Celgene for manufacturing services. The $109.0 million total allocable arrangement consideration was allocated based on the relative estimated selling price of the separate units of accounting at the inception of the amended agreement, resulting in $17.3 million allocated to the three delivered elements at the inception of the agreement, which will be recognized over an initial three year term.

The Company is required to reassess its conclusions on standalone value of deliverables upon delivery, and therefore, upon Celgene’s exercise of its option to obtain an exclusive worldwide license to develop and commercialize bb2121 in February 2016, the Company updated its assessment. The Company determined that there were no changes in standalone value of the research and development services as the option was previously determined to be non-substantive, the Company continues to have an obligation to provide research and development services for bb2121 and other product candidates, and this obligation is separate and unrelated to the execution of the license agreement. Participation on the JSC and participation on the patent committee also continue to have standalone value from the other undelivered elements as there has been no change in facts that would change this conclusion. Accordingly, each of these three deliverables continues to qualify as a separate unit of accounting.

The Company determined that each of the identified deliverables that qualify as a separate unit of accounting continue to have the same period of performance (the three year term through projected initial Phase I clinical study substantial completion) and the same pattern of revenue recognition, ratably over the period of performance as there is no other discernible pattern of recognition, and therefore there is no change in the recognition of $17.3 million allocated to these three elements. As of September 30, 2016, this will continue to be recognized over a three year term that began in June 2015.

However, the Company concluded that the license to bb2121 does not have standalone value from one of the undelivered elements, the post-initial Phase I the manufacture of vectors and associated payload for bb2121 under the license, because the manufacturing is essential to the license agreement. Accordingly, these two deliverables qualify as a single combined unit of accounting.

The single combined unit of accounting comprised of the license to bb2121 and the manufacture of vectors and associated payload for bb2121 were allocated consideration of $91.7 million, which will begin to be recognized upon the commencement of manufacturing services for bb2121 for Celgene post-initial Phase I, not in excess of the fixed consideration and assuming other revenue recognition criteria have been met. The Company currently expects this to commence in the second half of 2017 or first half of 2018. Revenue for the combined unit of account will be recognized on a proportional performance method or ratably over the period of performance if there is no other discernible pattern of recognition. This period of performance and recognition pattern will be revisited as the development plan changes or if other events impacting the deliverables occur.

The Company 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

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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 that may be received under the option to the license agreement 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 months ended September 30, 2016 and 2015, the Company recognized $1.6 million and $1.3 million, respectively, of revenue associated with its collaboration with Celgene related to the recognition of discovery, research and development services. During the nine months ended September 30, 2016 and 2015 the Company recognized $4.6 million and $12.6 million, respectively, of revenue associated with its same collaboration. As of September 30, 2016 and December 31, 2015, there was $48.0 million and $41.8 million, respectively, of total deferred revenue related to the Company's collaboration with Celgene, which is classified as current or non-current in the condensed consolidated balance sheets, $10.3 million of which is currently expected to be recognized through the first half of 2018 with the remaining amount deferred until a later date, as described above.

9. Stock-based compensation

In January 2016, the number of shares of common stock available for issuance under the 2013 Stock Option and Incentive Plan ("2013 Plan") was increased by approximately 1.5 million shares as a result of the automatic increase provision of the 2013 Plan. As of September 30, 2016, the total number of shares of common stock available for issuance under the 2013 Plan was approximately 1.2 million.

Stock-based compensation expense

Stock-based compensation expense by award type included within the condensed consolidated statements of operations and comprehensive loss was as follows (in thousands):

	Three months ended		Nine months ended	
	September 30,		September 30,	
	2016	2015	2016	2015
Stock options	$8,294	$8,407	$26,278	$28,472

Restricted stock units	1,495	1,056	4,248	2,341
Employee stock purchase plan	116	66	305	198
	$9,905	$9,529	$30,831	$31,011

As of September 30, 2016, the Company had $89.4 million of unrecognized stock-based compensation expense, net of estimated forfeitures, related to unvested stock options, restricted stock units and the employee stock purchase plan, which is expected to be recognized over a weighted-average period of 2.6 years.

Stock-based compensation expense by classification included within the condensed consolidated statements of operations and comprehensive loss was as follows (in thousands):

	Three months ended		Nine months ended	
	September 30,		September 30,	
	2016	2015	2016	2015
Research and development	$5,399	$4,426	$15,410	$19,726
General and administrative	4,506	5,103	15,421	11,285
	$9,905	$9,529	$30,831	$31,011

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In the first quarter of 2015, the Company modified outstanding options held by its former Chief Scientific Officer in connection with the termination of his employment. The incremental value of the modification was $3.0 million. As a result of the modification, the Company recognized $0.2 million and $3.0 million of stock-based compensation expense during the three and nine months ended September 30, 2015, respectively.

In the second quarter of 2015, the Company modified the vesting conditions of a stock option award held by a non-employee founder, which resulted in $6.7 million of stock-based compensation expense recognized to research and development expense during the second quarter of 2015.

Stock options

The following table summarizes the stock option activity under the Company's equity award plans (shares in thousands):

	Shares	Weighted-average exercise price per share
Outstanding at December 31, 2015	3,532	$ 48.74
Granted	944	$ 50.55
Exercised	(273)	$ 11.71
Canceled or forfeited	(336)	$ 48.88
Outstanding at September 30, 2016	3,867	$ 51.74
Exercisable at September 30, 2016	1,850	$ 38.04
Vested and expected to vest at September 30, 2016	3,776	$ 51.53

Options exercisable for 0.3 million shares of common stock were exercised during the nine months ended September 30, 2016, resulting in total proceeds to the Company of $3.2 million. In accordance with the Company's equity award plans, the shares were issued from a pool of shares reserved for issuance under the equity award plans.

Restricted stock units

The following table summarizes the restricted stock unit activity under the Company's equity award plans (shares in thousands):

	Shares	Weighted-average grant date fair value
Unvested balance at December 31, 2015	148	$ 65.79
Granted	236	$ 50.55
Vested	(110)	$ 45.57
Forfeited	(20)	$ 41.63
Unvested balance at September 30, 2016	254	$ 62.23

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 IPO on June 24, 2013. The 2013 ESPP authorizes the initial issuance of up to a total of 238,000 shares of the Company's common stock to participating employees. The first offering period under the 2013 ESPP opened on August 1, 2014. During the nine months ended September 30, 2016 and 2015, 18,338 shares and 10,545 shares of common stock were issued under the 2013 ESPP, respectively.

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10. 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. 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.

For the three and nine months ended September 30, 2016, the Company recognized an income tax benefit of $0.2 and $0.5 million and an income tax (benefit) expense in other comprehensive loss of $(0.1) and $0.8 million related to the unrealized gain (loss) on available-for-sale securities. As of September 30, 2016, the Company recorded an accrued income tax provision of $0.2 million related to this tax benefit included within accrued expenses and other current liabilities in the condensed consolidated balance sheet, which is expected to be generated from continuing operations.

11. Net loss per share

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

	For the three and nine months ended September 30,	
	2016	2015
Outstanding stock options	3,867	3,661
Restricted stock units	254	149
ESPP shares	12	3
Acquisition holdback	—	94
	4,133	3,907

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 our Annual Report on Form 10-K, which was filed with the Securities and Exchange Commission, or the SEC, on February 25, 2016.

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," "expect," "anticipate," "estimate," "intend," "plan," 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 committed to developing potentially transformative gene therapies for severe genetic and rare diseases and in the field of T cell-based immunotherapy. With our lentiviral-based gene therapy and gene editing capabilities, we have built an integrated product platform with broad potential application in these areas. We believe that gene therapy for severe genetic diseases 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 treatments 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 three underserved diseases.

We are conducting four clinical studies of our LentiGlobin product candidate: a Phase I/II study in the United States, Australia, and Thailand, called the Northstar Study, for the treatment of transfusion-dependent -thalassemia, or TDT; a global, multi-center Phase III study called the Northstar-2 Study, for the treatment of patients with TDT who do not have a $^{0}/^{0}$ genotype; a single-center Phase I/II study in France (HGB-205) for the treatment of TDT and severe sickle cell disease, or severe SCD; and a Phase I study in the United States (HGB-206) for the treatment of severe SCD. We have achieved our enrollment target of 18 patients in the Northstar Study. Both TDT and severe SCD are rare, hereditary blood disorders that often lead to severe anemia and shortened lifespans. Our LentiGlobin product candidate has been granted Orphan Drug status by the U.S. Food and Drug Administration, or FDA, and the European

Medicines Agency, or EMA, for both -thalassemia and SCD. Our LentiGlobin product candidate was granted Fast-Track designation by the FDA for the treatment of -thalassemia major in January 2013 and for the treatment of certain patients with severe SCD in May 2014. In January 2015, the FDA granted Breakthrough Therapy designation to our LentiGlobin product candidate for the treatment of transfusion-dependent patients with -thalassemia major. In September 2016, the EMA has granted access to its Priority Medicines (PRIME) scheme for our LentiGlobin product candidate for the treatment of TDT. Also in September 2016, we initiated our NorthStar-2 Study, which we expect will enroll approximately 15 adult and adolescent patients with TDT who do not have a $^{0}/^{0}$ genotype, with an additional pediatric cohort of up to eight patients for a total enrollment of approximately 23 patients. We expect to evaluate the patients for 24 months following treatment, and anticipate that the primary endpoint of this study will be 12 months of transfusion independence following treatment. We have discussed with the FDA and EMA the design of our planned Phase III study (HGB-212) of our LentiGlobin product candidate for patients with TDT who have a $^{0}/^{0}$ genotype, and we anticipate that the primary endpoint of this study will be transfusion reduction.

We are also conducting a Phase II/III clinical study, called the Starbeam Study, of our Lenti-D product candidate, to evaluate its safety and efficacy in subjects with cerebral adrenoleukodystrophy, or CALD, a rare, hereditary neurological disorder that is often fatal. In October 2013, we announced that the first subject had been treated in this study and in May 2015 we announced the achievement of enrollment of 18 subjects in this study. We are also conducting an observational study of subjects with CALD treated by allogeneic hematopoietic stem-cell transplant referred to as the ALD-103 study. Our Lenti-D product candidate has been granted Orphan Drug status by the FDA and the EMA for the treatment of adrenoleukodystrophy.

In March 2013, we entered into a global strategic collaboration with Celgene Corporation, or Celgene, to discover, develop and commercialize chimeric antigen receptor-modified T cells, or CAR T cells, as potentially disease-altering therapies in oncology. This collaboration had an initial term of three years, and Celgene made a $75.0 million up-front, non-refundable cash payment to us as consideration for entering into the collaboration. In June 2015, we amended and restated the collaboration agreement, or the Amended Collaboration Agreement, to focus exclusively on anti-BCMA product candidates for a new three-year term. B-cell maturation antigen, or BCMA, is a cell surface protein that is expressed on normal plasma cells and on most multiple myeloma cells, but is absent from other normal tissues. As consideration for the Amended Collaboration Agreement, we received an upfront, non-refundable cash payment of $25.0 million to fund research and development under the collaboration. In February 2016, we treated the first subject in our Phase I clinical study of bb2121, the first anti-BCMA product candidate from this collaboration. This study will enroll up to 40 patients who have received three prior regimens for treatment of multiple myeloma. In February 2016, Celgene exercised its option to obtain an exclusive worldwide license to develop and commercialize bb2121 and as a result, has paid to us an option fee in the amount of $10.0 million in the first quarter of 2016. We may elect to co-develop and co-promote bb2121, and any other product candidates in the United States under this collaboration arrangement. In May 2016, the FDA granted Orphan Drug status to our bb2121 product candidate for the treatment of multiple myeloma.

In June 2014, we acquired Precision Genome Engineering, Inc., or Pregenen, a privately-held biotechnology company headquartered in Seattle, Washington. Through the acquisition, we obtained rights to Pregenen's gene editing and cell signaling technology. The agreement provided for up to $135.0 million in future contingent cash payments by us upon the achievement of certain preclinical, clinical and commercial milestones related to the Pregenen technology, of which $15.0 million relates to preclinical milestones, $20.1 million relates to clinical milestones and $99.9 million relates to commercial milestones. We estimate future contingent cash payments have a fair value of $7.2 million as of September 30, 2016, $3.9 million of which is classified as a current liability.

As of September 30, 2016, we had cash, cash equivalents and marketable securities of approximately $727.6 million. We expect that our existing cash, cash equivalents and marketable securities will be sufficient to fund our current operations through 2018.

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 candidates in compliance with good manufacturing practices, or GMP, to conduct clinical studies of our product candidates, to provide general and administrative support for these operations and to protect 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 sale of common stock in our public offerings, private placements of preferred stock and warrants and through collaborations.

We have never been profitable and have incurred net losses in each year since inception. Our net loss was $192.1 million for the nine months ended September 30, 2016 and our accumulated deficit was $506.3 million as of September 30, 2016. Substantially all of 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 LentiGlobin, Lenti-D, and bb2121 product candidates;
- increase research and development-related activities for the discovery and development of oncology product candidates;
- continue our research and development efforts;
- manufacture clinical study materials and develop large-scale manufacturing capabilities;
- seek regulatory approval for our product candidates; and
- add personnel to support our product development and commercialization efforts.

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 and marketing organization. If we seek to obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses as we prepare for product sales, marketing, manufacturing, and distribution. Accordingly, we will seek to fund our operations through public or private equity or debt financings, strategic collaborations, 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.

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.

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, which was amended in 2015. The terms of this amended arrangement contain multiple deliverables, which include: (i) research and development services, (ii) participation on the joint steering committee, (iii) participation on the patent committee, (iv) a license to the first product candidate, (v) manufacture of vectors and associated payload for incorporation into the first optioned product candidate under the license, and (vi) participation on the joint governance committee under the co-development and co-promotion agreement for the first optioned product candidate under the license. 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. We expect that $17.3 million of revenue from the Celgene arrangement associated with discovery, research and development services, joint steering committee services and patent committee services will be recognized ratably over the associated period of performance, which was initially estimated to be three years from the date of the agreement in June 2015. We expect that $91.7 million of revenue, not in excess of the fixed consideration and assuming other revenue recognition criteria have been met, from the Celgene arrangement associated with the license to the first product candidate, bb2121, and the manufacture of vector and associated payload for bb2121 following the initial Phase I study will be recognized on a proportional performance method or ratably over the period of performance if there is no other discernible pattern of recognition. This period of performance and recognition pattern will be revisited as the development plan changes or if other events impacting the deliverables occur.

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 clinical sites that 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, information technology, and other supplies;
- costs associated with our research platform and preclinical activities;
- costs associated with in-licensing other product candidates or technologies for use in preclinical and clinical activities;
- costs associated with our regulatory, quality assurance and quality control operations; and
- amortization of intangible assets.

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 we undertake;
- future clinical study results;
- uncertainties in clinical study enrollment rates;
- changing standards for regulatory approval; 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 for our product candidates.

We plan to increase our research and development expenses for the foreseeable future as we continue to advance the clinical development of our Lenti-D and LentiGlobin product candidates, conduct research and development activities in the field of oncology and continue the research and development of product candidates using our gene editing technology platform. Our research and development activities include the following:

- We are conducting a Phase II/III clinical study to examine the safety and efficacy of our Lenti-D product candidate in the treatment of CALD. In October 2013, we announced that the first subject had been treated in this study and in May 2015 we announced the achievement of enrollment of 18 subjects in this study. We are also conducting an observational study of subjects with CALD treated by allogeneic hematopoietic stem-cell transplant.
- We are conducting a Phase I/II clinical study in the United States, Australia and Thailand to study the safety and efficacy of our LentiGlobin product candidate in the treatment of subjects with TDT. In March 2014, we announced that the first subject had been treated in this study.
- We are conducting a Phase I/II clinical study in France to study the safety and efficacy of our LentiGlobin product candidate in the treatment of subjects with TDT and severe SCD. In December 2013, we announced that the first subject with TDT had been treated in this study and in October 2014, we announced that the first subject with severe SCD had been treated in this study.
- We are conducting a Phase I clinical study in the United States to study the safety and efficacy of our LentiGlobin product candidate in the treatment of subjects with severe SCD. In June 2015, we announced that the first patient with severe SCD had been treated in this study.
- We are conducting a global, multi-center Phase III clinical study to study the safety and efficacy of our LentiGlobin product candidate in the treatment of patients with a diagnosis of TDT who have non-$^{0}/^{0}$ genotypes.
- We are planning to initiate in 2017, a global, multi-center Phase III clinical study to study the safety and efficacy of our LentiGlobin product candidate in the treatment of patients with a diagnosis of TDT who have $^{0}/^{0}$ genotypes.
- We are conducting a Phase I clinical study in the United States to study the safety and efficacy of our bb2121 product candidate in the treatment of subjects with relapsed/refractory multiple myeloma. In February 2016, we announced that the first subject with relapsed/refractory multiple myeloma had been treated in this study.
- 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 fees paid to investigators, consultants, central laboratories and CROs in connection with our clinical studies, costs to in-license product candidates and new technologies, and costs related to acquiring and manufacturing clinical study materials. We allocate salary and benefit costs directly related to specific programs. We do not allocate personnel-related discretionary bonus or stock-based compensation costs, costs associated with our general discovery platform improvements, depreciation or other indirect costs that are deployed across multiple projects under development and, as such, the costs are separately classified as personnel and other expenses in the table below:

	Three months ended		Nine months ended	
	September 30,		September 30,	
	2016	2015	2016	2015
	(in thousands)		(in thousands)	
LentiGlobin	$16,154	$10,937	$45,898	$25,070
Lenti-D	5,663	2,837	10,956	10,729
bb2121	3,613	—	8,900	—
Pre-clinical programs	20,856	3,680	29,378	11,596
Total direct research and development expense	46,286	17,454	95,132	47,395
Employee-and contractor-related expenses	4,015	3,568	12,401	9,029
Stock-based compensation expense	5,399	4,426	15,410	19,726
Platform-related expenses	2,767	2,985	9,178	17,047
Facility expenses	5,209	1,724	14,606	4,710
Other expenses	295	238	915	473
Unallocated personnel and other expenses				