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 June 30, 2020

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.)**
60 Binney Street	
Cambridge , Massachusetts	**02142**
(Address of Principal Executive Offices)	**(Zip Code)**

(339) 499-9300

(Registrant's Telephone Number, Including Area Code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.01 par value per share	BLUE	The NASDAQ Stock Market LLC

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 every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

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 July 31, 2020, there were 66,222,965 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,” “seek,” “should,” “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, and to ensure adequate supply of our viral vectors and drug products;
- the timing or likelihood of regulatory filings and approvals for our product candidates;
- the timing or success of commercialization of our approved product, and any future approved products;
- the pricing and reimbursement of our approved product, and any future approved products;
- 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 approved product, 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;
- the impact of the COVID-19 pandemic; 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.

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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)

	As of June 30, 2020	As of December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 1,198,768	$ 327,214
Marketable securities	350,614	779,246
Prepaid expenses	39,358	32,888
Receivables and other current assets	24,705	12,826
Total current assets	1,613,445	1,152,174
Marketable securities	49,411	131,506
Property, plant and equipment, net	155,376	151,176
Intangible assets, net	12,183	14,326
Goodwill	13,128	13,128
Operating lease right-of-use assets	189,464	185,885
Restricted cash and other non-current assets	74,783	79,229
Total assets	$ 2,107,790	$ 1,727,424
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 26,181	$ 42,995
Accrued expenses and other current liabilities	135,612	141,556
Operating lease liability, current portion	20,955	20,175
Deferred revenue, current portion	3,915	8,474
Collaboration research advancement, current portion	10,518	10,380
Total current liabilities	197,181	223,580
Deferred revenue, net of current portion	25,762	9,791
Collaboration research advancement, net of current portion	23,917	27,834
Operating lease liability, net of current portion	174,564	170,812
Other non-current liabilities	4,335	10,414
Total liabilities	425,759	442,431
Commitments and contingencies *(Note 8)*		
Stockholders' equity:		
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at June 30, 2020 and December 31, 2019	—	—
Common stock, $0.01 par value, 125,000 shares authorized; 66,196 and 55,368 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	662	554
Additional paid-in capital	4,189,697	3,568,184
Accumulated other comprehensive loss	(2,400)	(1,893)
Accumulated deficit	(2,505,928)	(2,281,852)
Total stockholders' equity	1,682,031	1,284,993
Total liabilities and stockholders' equity	$ 2,107,790	$ 1,727,424

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)

	For the three months ended June 30,		For the six months ended June 30,	
	2020	2019	2020	2019
Revenue:				
Service revenue	$ 78,357	$ 11,093	$ 95,190	$ 20,304
Collaborative arrangement revenue	109,674	465	111,976	2,431
Royalty and other revenue	10,859	1,738	13,587	3,032
Total revenues	198,890	13,296	220,753	25,767
Operating expenses:				
Research and development	156,308	146,540	310,431	269,180
Selling, general and administrative	68,628	68,631	141,876	128,910
Cost of royalty and other revenue	1,554	613	2,579	1,043
Change in fair value of contingent consideration	(1,655)	214	(4,763)	510
Total operating expenses	224,835	215,998	450,123	399,643
Loss from operations	(25,945)	(202,702)	(229,370)	(373,876)
Interest income, net	2,939	9,387	8,294	19,489
Other income (expense), net	1,551	(2,936)	(2,896)	(6,325)
Loss before income taxes	(21,455)	(196,251)	(223,972)	(360,712)
Income tax (expense) benefit	(10)	469	(104)	484
Net loss	$ (21,465)	$ (195,782)	$ (224,076)	$ (360,228)
Net loss per share - basic and diluted:	$ (0.36)	$ (3.55)	$ (3.86)	$ (6.54)
Weighted-average number of common shares used in computing net loss per share - basic and diluted:	60,384	55,165	57,987	55,062
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax expense of $0.1 million and $0.8 million for the three months ended June 30, 2020 and 2019, respectively, and $0.1 million and $1.3 million for the six months ended June 30, 2020 and 2019, respectively	399	973	(507)	2,808
Total other comprehensive income (loss)	399	973	(507)	2,808
Comprehensive loss	$ (21,066)	$ (194,809)	$ (224,583)	$ (357,420)

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Condensed Consolidated Statements of Stockholders' Equity
(unaudited)
(in thousands)

	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balances at December 31, 2019	55,368	$ 554	$ 3,568,184	$ (1,893)	$ (2,281,852)	$ 1,284,993
Vesting of restricted stock units	204	2	(2)	—	—	—
Exercise of stock options	20	—	750	—	—	750
Purchase of common stock under ESPP	28	—	1,872	—	—	1,872
Stock-based compensation	—	—	36,335	—	—	36,335
Other comprehensive loss	—	—	—	(906)	—	(906)
Net loss	—	—	—	—	(202,611)	(202,611)
Balances at March 31, 2020	55,620	$ 556	$ 3,607,139	$ (2,799)	$ (2,484,463)	$ 1,120,433
Issuance of common stock upon public offering, net of issuance costs of $33,465	10,455	105	541,431	—	—	541,536
Vesting of restricted stock units	114	1	(1)	—	—	—
Exercise of stock options	7	—	347	—	—	347
Stock-based compensation	—	—	40,781	—	—	40,781
Other comprehensive income	—	—	—	399	—	399
Net loss	—	—	—	—	(21,465)	(21,465)
Balances at June 30, 2020	66,196	$ 662	$ 4,189,697	$ (2,400)	$ (2,505,928)	$ 1,682,031

	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balances at December 31, 2018	54,738	$ 547	$ 3,386,958	$ (3,627)	$ (1,498,808)	$ 1,885,070
Adjustments to beginning accumulated deficit from adoption of ASU 2016-02	—	—	—	—	6,564	6,564
Vesting of restricted stock units	131	2	(2)	—	—	—
Exercise of stock options	189	2	9,502	—	—	9,504
Purchase of common stock under ESPP	11	—	1,231	—	—	1,231
Stock-based compensation	—	—	32,341	—	—	32,341
Other comprehensive income	—	—	—	1,835	—	1,835
Net loss	—	—	—	—	(164,446)	(164,446)
Balances at March 31, 2019	55,069	$ 551	$ 3,430,030	$ (1,792)	$ (1,656,690)	$ 1,772,099
Vesting of restricted stock units	66	$ 1	$ (1)	$ —	$ —	$ —
Exercise of stock options	93	1	3,972	—	—	3,973
Stock-based compensation	—	—	55,111	—	—	55,111
Other comprehensive income	—	—	—	973	—	973
Net loss	—	—	—	—	(195,782)	(195,782)
Balances at June 30, 2019	55,228	$ 553	$ 3,489,112	$ (819)	$ (1,852,472)	$ 1,636,374

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Condensed Consolidated Statements of Cash Flows
(unaudited)
(in thousands)

	For the six months ended June 30,	
	2020	**2019**
Cash flows from operating activities:		
Net loss	$ (224,076)	$ (360,228)
Adjustments to reconcile net loss to net cash used in operating activities:		
Change in fair value of contingent consideration	(4,763)	510
Depreciation and amortization	9,430	7,831
Stock-based compensation expense	84,822	87,452
Unrealized loss on equity securities	3,343	6,184
Other non-cash items	(1,841)	(7,064)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(13,813)	(20,694)
Operating lease right-of-use assets	11,085	11,037
Accounts payable	(14,042)	6,652
Accrued expenses and other liabilities	(14,025)	(9,301)
Operating lease liabilities	(10,131)	(259)
Deferred revenue	11,412	(11,716)
Collaboration research advancement	(3,779)	(2,431)
Net cash used in operating activities	(166,378)	(292,027)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(15,478)	(37,925)
Purchases of marketable securities	(101,421)	(471,365)
Sales of marketable securities	29,878	—
Proceeds from maturities of marketable securities	580,875	704,803
Net cash provided by investing activities	493,854	195,513
Cash flows from financing activities:		
Proceeds from public offering of common stock, net of issuance costs	541,536	—
Proceeds from exercise of stock options and ESPP contributions	2,549	15,004
Net cash provided by financing activities	544,085	15,004
Increase (decrease) in cash, cash equivalents and restricted cash	871,561	(81,510)
Cash, cash equivalents and restricted cash at beginning of period	381,709	417,099
Cash, cash equivalents and restricted cash at end of period	$ 1,253,270	$ 335,589
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 1,198,768	$ 280,995
Restricted cash included in receivables and other current assets	$ —	$ 100
Restricted cash included in restricted cash and other non-current assets	$ 54,502	$ 54,494
Total cash, cash equivalents and restricted cash	$ 1,253,270	$ 335,589
Supplemental cash flow disclosures from investing and financing activities:		
Purchases of property, plant and equipment included in accounts payable and accrued expenses	$ 1,257	$ 8,869
Right-of-use assets obtained in exchange for operating lease liabilities	$ 14,663	$ 17,489

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 is a biotechnology company committed to researching, developing and commercializing potentially transformative gene therapies for severe genetic diseases and cancer. 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 selling, general and administrative support for these operations, including commercial-readiness activities.

The Company’s programs in severe genetic diseases include betibeglogene autotemcel (beti-cel; formerly LentiGlobin for β-thalassemia gene therapy) as a treatment for transfusion-dependent β-thalassemia, or TDT; its LentiGlobin® product candidate as a treatment for sickle cell disease, or SCD; and elivaldogene autotemcel (eli-cel; formerly Lenti-D gene therapy) as a treatment for cerebral adrenoleukodystrophy, or CALD. The Company’s programs in oncology are focused on developing novel T cell-based immunotherapies, including chimeric antigen receptor (CAR) and T cell receptor (TCR) T cell therapies. Idecabtagene vicleucel, or ide-cel, and bb21217, are product candidates in oncology under the Company’s collaboration arrangement with Bristol-Myers Squibb ("BMS"), formerly Celgene Corporation ("Celgene") prior to its acquisition by BMS in November 2019. ide-cel and bb21217 are CAR T cell product candidates for the treatment of multiple myeloma. Please refer to Note 9, *Collaborative arrangements*, for further discussion of the Company’s collaboration with BMS.

In June 2019, the Company received conditional marketing authorization from the European Commission for beti-cel as a treatment of patients 12 years and older with TDT who do not have a β^0/β^0 genotype, for whom hematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte-matched related HSC donor is not available. beti-cel is being marketed as ZYNTEGLO™ in the European Union. Through June 30, 2020, the Company had not generated any revenue from product sales of ZYNTEGLO.

As of June 30, 2020, the Company had cash, cash equivalents and marketable securities of $1.60 billion. The Company expects that its cash, cash equivalents and marketable securities will be sufficient to fund current planned operations for at least twelve months from the date of issuance of these financial statements, though it may pursue additional cash resources through public or private debt and equity financings and establish collaborations with or license its technology to other companies.

2. Basis of presentation, principles of consolidation and significant accounting policies

Basis of presentation

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 Updates (“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 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 June 30, 2020 and 2019.

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 condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2019, 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 18, 2020.

Certain items in the prior year’s condensed consolidated financial statements have been reclassified to conform to the current presentation. However, no subtotals in the prior year condensed consolidated financial statements were impacted as a result.

Amounts reported are computed based on thousands. As a result, certain totals may not sum due to rounding.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. 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.

Significant accounting policies

The significant accounting policies used in preparation of these condensed consolidated financial statements for the three and six months ended June 30, 2020 are consistent with those discussed in Note 2 to the consolidated financial statements included in the Company's 2019 Annual Report on Form 10-K, except as noted immediately below and as noted within the *"Recent accounting pronouncements - Recently adopted"* section.

Marketable securities

Effective January 1, 2020, the Company adopted ASU 2016-13, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Statements* ("ASU 2016-13" or "ASC 326"), using the effective date method. As the Company had never recorded any other-than-temporary-impairment adjustments to its available-for-sale debt securities prior to the effective date, no transition provisions are applicable to the Company.

The Company assesses its available-for-sale debt securities under the available-for-sale debt security impairment model in ASC 326 as of each reporting date in order to determine if a portion of any decline in fair value below carrying value recognized on its available-for-sale debt securities is the result of a credit loss. The Company records credit losses in the condensed consolidated statements of operations and comprehensive loss as credit loss expense within other expense, net, which is limited to the difference between the fair value and the amortized cost of the security. To date, the Company has not recorded any credit losses on its available-for-sale debt securities.

Accrued interest receivable related to the Company's available-for-sale debt securities is presented within receivables and other current assets on the Company's condensed consolidated balance sheets. The Company has elected the practical expedient available to exclude accrued interest receivable from both the fair value and the amortized cost basis of available-for-sale debt securities for the purposes of identifying and measuring any impairment. The Company writes off accrued interest receivable once it has determined that the asset is not realizable. Any write offs of accrued interest receivable are recorded by reversing interest income, recognizing credit loss expense, or a combination of both. To date, the Company has not written off any accrued interest receivables associated with its marketable securities.

Stock-based compensation

The Company estimates the fair value of its option awards using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including (i) the expected stock price volatility, (ii) the calculation of the expected term of the award, (iii) the risk-free interest rate, and (iv) expected dividends. Effective January 1, 2020, the Company eliminated the use of a representative peer group and uses only its own historical volatility data in its estimate of expected volatility given that there is now a sufficient amount of historical information regarding the volatility of its own stock price.

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: future undiscounted cash flows and subsequent fair value estimates used to assess potential and measure any impairment of long-lived assets, including goodwill and intangible assets, and the measurement of right-of-use assets and lease liabilities, contingent consideration, stock-based compensation expense, accrued expenses, revenue, income taxes, and the assessment of the Company's ability to fund its operations for at least the next twelve months from the date of issuance of these financial statements.

Recent accounting pronouncements

Recently adopted

ASU No. 2016-13, Financial Instruments – Credit Losses (Topic 326): Measurement of Credit Losses on Financial Statements, ASU No. 2019-5 Financial Instruments – Credit Losses (Topic 326): Targeted Transition Relief, ASU No. 2019-11, Codification Improvements to Topic 326, Financial Instruments - Credit Losses

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments – Credit Losses (Topic 326): Measurement of Credit Losses on Financial Statements*. The new standard, as amended, requires that expected credit losses relating to financial assets measured on an amortized cost basis and available-for-sale debt securities be recorded through an allowance for credit losses. It also limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and also requires the reversal of previously recognized credit losses if fair value increases. The targeted transition relief standard allows filers an option to irrevocably elect the fair value option of ASC 825-10, *Financial Instruments-Overall*, applied on an instrument-by-instrument basis for eligible instruments. The Company adopted this standard on January 1, 2020 on a prospective basis and the adoption did not have a material impact on its financial position and results of operations.

ASU No. 2018-13, Fair Value Measurement (Topic 820): Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement.* The new standard removes certain disclosures, modifies certain disclosures, and adds additional disclosures related to fair value measurement. The Company adopted this standard on January 1, 2020, and it did not have a material impact on its financial position and results of operations upon adoption.

ASU No. 2018-15, Intangibles-Goodwill and Other - Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract

In August 2018, the FASB issued ASU 2018-15, *Intangibles-Goodwill and Other-Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract.* The amendments in this update align the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). The accounting for the service element of a hosting arrangement that is a service contract is not affected by the amendments in this update. The Company adopted this standard on a prospective basis as of January 1, 2020, and it did not have a material impact on its financial position and results of operations upon adoption.

ASU No. 2018-18, Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606

In November 2018, the FASB issued ASU 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606*, ("ASU 2018-18"). The amendments in this update clarify that certain transactions between collaborative arrangement participants should be accounted for as revenue under ASC 606, *Revenue from Contracts with Customers* ("Topic 606" or "ASC 606") when the counter party is a customer in the context of a unit of account. ASU 2018-18 also precludes companies from presenting transactions with collaborative partners that are outside the scope of Topic 606 together with revenue within the scope of Topic 606. The Company adopted this standard on a retrospective basis on January 1, 2020. As a result, revenue for prior periods are presented in accordance with the new standard.

Prior to the adoption of ASU 2018-18, the Company presented all revenue recognized under its collaborative arrangements as collaboration revenue on its condensed consolidated statement of operations and comprehensive loss. However, as the Company recognizes revenue under its collaborative arrangements both within and outside the scope of Topic 606, the Company has revised its presentation of revenue on its condensed consolidated statement of operations and comprehensive loss as follows: service revenue includes revenue from collaborative partners recognized within the scope of Topic 606 and collaborative arrangement revenue includes revenue from collaborative partners recognized outside the scope of Topic 606. The disaggregation of revenue recognized under Topic 606 and outside of Topic 606 had previously otherwise been disclosed in the Notes to Condensed Consolidated Financial Statements.

ASU No. 2019-4, Codification Improvements to Topic 326, Financial Instruments – Credit Losses, Topic 815, Derivatives and Hedging, and Topic 825, Financial Instruments

In April 2019, the FASB issued ASU 2019-4, *Codification Improvements to Topic 326, Financial Instruments – Credit Losses, Topic 815, Derivatives and Hedging, and Topic 825, Financial Instruments.* This update provides clarifications for three topics related to financial instruments accounting, some of which apply to the Company. The Company adopted this standard on January 1, 2020 on a prospective basis, and it did not have a material impact on its financial position and results of operations upon adoption.

Not yet adopted

ASU No. 2019-12, Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes* ("ASU 2019-12"), which is intended to simplify the accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in Topic 740 and also clarifies and amends existing guidance to improve consistent application. The new standard will be effective beginning January 1, 2021. The Company is currently evaluating the potential impact ASU 2019-12 may have on its financial position and results of operations upon adoption.

3. Marketable securities

The following table summarizes the marketable securities held at June 30, 2020 and December 31, 2019 (in thousands):

Description	Amortized cost / Cost	Unrealized gains	Unrealized losses	Fair value
June 30, 2020				
U.S. government agency securities and treasuries	$ 201,362	$ 1,294	$ (5)	$ 202,651
Corporate bonds	151,305	1,620	—	152,925
Commercial paper	34,922	—	—	34,922
Equity securities	20,017	—	(10,490)	9,527
Total	$ 407,606	$ 2,914	$ (10,495)	$ 400,025
December 31, 2019				
U.S. government agency securities and treasuries	$ 633,970	$ 2,014	$ (48)	$ 635,936
Certificates of deposit	960	—	—	960
Corporate bonds	185,827	824	(43)	186,608
Commercial paper	74,378	—	—	74,378
Equity securities	20,017	—	(7,147)	12,870
Total	$ 915,152	$ 2,838	$ (7,238)	$ 910,752

No available-for-sale debt securities held as of June 30, 2020 or December 31, 2019 had remaining maturities greater than five years.

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 June 30, 2020 and December 31, 2019 (in thousands):

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
June 30, 2020				
Assets:				
Cash and cash equivalents	$ 1,198,768	$ 1,198,768	$ —	$ —
Marketable securities:				
U.S. government agency securities and treasuries	202,651	—	202,651	—
Corporate bonds	152,925	—	152,925	—
Commercial paper	34,922	—	34,922	—
Equity securities	9,527	9,527	—	—
Total	$ 1,598,793	$ 1,208,295	$ 390,498	$ —
Liabilities:				
Contingent consideration	$ 3,214	$ —	$ —	$ 3,214
Total	$ 3,214	$ —	$ —	$ 3,214
December 31, 2019				
Assets:				
Cash and cash equivalents	$ 327,214	$ 311,245	$ 15,969	$ —
Marketable securities:				
U.S. government agency securities and treasuries	635,936	—	635,936	—
Certificates of deposit	960	—	960	—
Corporate bonds	186,608	—	186,608	—
Commercial paper	74,378	—	74,378	—
Equity securities	12,870	12,870	—	—
Total	$ 1,237,966	$ 324,115	$ 913,851	$ —
Liabilities:				
Contingent consideration	$ 7,977	$ —	$ —	$ 7,977
Total	$ 7,977	$ —	$ —	$ 7,977

Cash and cash equivalents

The Company considers all highly liquid securities with original final maturities of 90 days or less from the date of purchase to be cash equivalents. As of June 30, 2020, cash and cash equivalents comprise funds in cash and money market accounts. As of December 31, 2019, cash and cash equivalents comprise funds in cash, money market accounts, and commercial paper.

Marketable securities

Marketable securities classified as Level 2 within the valuation hierarchy generally consist of certificates of deposit, U.S. treasury securities and government agency securities, corporate bonds, and commercial paper. The Company estimates the fair values of these marketable securities by taking into consideration valuations obtained from third-party pricing sources. These pricing sources utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include market pricing based on real-time trade data for the same or similar securities, issuer credit spreads, benchmark yields, and other observable inputs. The Company validates the prices provided by its third-party pricing sources by understanding the models used, obtaining market values from other pricing sources and analyzing pricing data in certain instances.

The amortized cost of available-for-sale debt securities is adjusted for amortization of premiums and accretion of discounts to the earliest call date for premiums or to maturity for discounts. At June 30, 2020 and December 31, 2019, the balance in the

Company's accumulated other comprehensive loss includes activity related to the Company's available-for-sale debt securities. There were no material realized gains or losses recognized on the sale or maturity of available-for-sale securities during the three and six months ended June 30, 2020 or 2019.

Accrued interest receivable on the Company's available-for-sale debt securities totaled $1.7 million and $3.6 million as of June 30, 2020 and December 31, 2019, respectively. No accrued interest receivable was written off during the three and six months ended June 30, 2020 or 2019.

The following table summarizes available-for-sale debt securities in a continuous unrealized loss position for less than and greater than twelve months, and for which an allowance for credit losses has not been recorded at June 30, 2020 and December 31, 2019 (in thousands):

	Less than 12 months		12 months or greater		Total	
Description	**Fair value**	**Unrealized losses**	**Fair value**	**Unrealized losses**	**Fair value**	**Unrealized losses**
June 30, 2020						
U.S. government agency securities and treasuries	$ —	$ —	$ 10,498	$ (5)	$ 10,498	$ (5)
Total	$ —	$ —	$ 10,498	$ (5)	$ 10,498	$ (5)
December 31, 2019						
U.S. government agency securities and treasuries	$ 13,234	$ (3)	$ 79,618	$ (45)	$ 92,852	$ (48)
Corporate bonds	53,983	(43)	—	—	53,983	(43)
Total	$ 67,217	$ (46)	$ 79,618	$ (45)	$ 146,835	$ (91)

The Company determined that there was no material change in the credit risk of the above investments during the six months ended June 30, 2020. As such, an allowance for credit losses was not recognized. As of June 30, 2020, the Company does not intend to sell such securities and it is not more likely than not that the Company will be required to sell the securities before recovery of their amortized cost bases.

The Company holds equity securities with an aggregate fair value of $9.5 million and $12.9 million as of June 30, 2020 and December 31, 2019, respectively, within short-term marketable securities on its condensed consolidated balance sheets. The Company has recorded an unrealized gain of $1.2 million and an unrealized loss of $3.3 million during the three and six months ended June 30, 2020, respectively, and unrealized losses of $3.1 million and $6.2 million during the three and six months ended June 30, 2019, respectively, related to its equity securities, which is included in other income (expense), net on the condensed consolidated statements of operations and comprehensive loss.

Contingent consideration

In connection with its prior acquisition of Precision Genome Engineering, Inc. ("Pregenen"), the Company may be required to pay future consideration that is contingent upon the achievement of specified development, regulatory approvals or sales-based milestone events. 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. In the absence of new information, changes in fair value will reflect changing discount rates and the passage of time. Contingent consideration is included in accrued expenses and other current liabilities and other non-current liabilities on the condensed consolidated balance sheets.

Please refer to Note 8, *Commitments and contingencies*, for further information.

5. Property, plant and equipment, net

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

	As of June 30, 2020	As of December 31, 2019
Land	$ 1,210	$ 1,210
Building	15,745	15,664
Computer equipment and software	6,837	6,947
Office equipment	7,611	7,599
Laboratory equipment	47,496	44,560
Leasehold improvements	34,019	33,788
Construction-in-progress	86,260	77,981
Total property, plant and equipment	199,178	187,749
Less accumulated depreciation and amortization	(43,802)	(36,573)
Property, plant and equipment, net	$ 155,376	$ 151,176

North Carolina manufacturing facility

In November 2017, the Company acquired a manufacturing facility, which is in the process of construction, in Durham, North Carolina for the future manufacture of lentiviral vector for the Company's gene therapies. As of June 30, 2020, a portion of the facility has been placed into service, and the remainder of the facility is still in process of construction. Construction-in-progress as of June 30, 2020 and December 31, 2019 includes $81.7 million and $74.2 million, respectively, related to the North Carolina manufacturing facility.

6. Accrued expenses and other current liabilities

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

	As of June 30, 2020	As of December 31, 2019
Employee compensation	$ 39,018	$ 44,679
Manufacturing costs	33,171	23,126
Clinical and contract research organization costs	21,577	16,799
Collaboration research costs	7,886	27,142
Property, plant, and equipment	685	2,354
License and milestone fees	771	300
Professional fees	1,847	1,827
Other	30,657	25,329
Total accrued expenses and other current liabilities	$ 135,612	$ 141,556

7. Leases

The Company leases certain office and laboratory space. Additionally, the Company has embedded leases at contract manufacturing organizations. Effective January 1, 2019, the Company adopted ASU 2016-02, Leases (Topic 842), ("ASC 842"), using the required modified retrospective approach and utilizing the effective date as the date of initial application.

60 Binney Street Lease

In September 2015, the Company entered into a lease agreement for office and laboratory space located in a building (the "Building") at 60 Binney Street, Cambridge, Massachusetts (the "60 Binney Street Lease"), which is now the Company's corporate headquarters. Under the terms of the 60 Binney Street Lease, starting on October 1, 2016, the Company leases 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

currently maintains a $13.8 million collateralized letter of credit and, subject to the terms of the lease and certain reduction requirements specified therein, including market capitalization requirements, this amount may decrease to $9.2 million over time. Pursuant to a work letter entered into in connection with the 60 Binney Street Lease, the landlord contributed an aggregate of $42.4 million toward the cost of construction and tenant improvements for the Building.

The Company occupied the Building beginning in March 2017 and the 60 Binney Street Lease will continue until March 31, 2027. The Company has the option to extend the 60 Binney Street Lease for two successive five-year terms. In applying the ASC 842 transition guidance, the Company classified this lease as an operating lease and recorded a right-of-use asset and lease liability on the effective date. The Company is recognizing rent expense on a straight-line basis throughout the remaining term of the lease.

50 Binney Street Sublease

In April 2019, the Company entered into a sublease agreement for office space located at 50 Binney Street in Cambridge, Massachusetts (the "50 Binney Street Sublease") to supplement the Company's corporate headquarters located at 60 Binney Street in Cambridge, Massachusetts. Under the terms of the 50 Binney Street Sublease, the Company will lease 267,278 square feet of office space for $99.95 per square foot, or $26.7 million per year in base rent subject to certain operating expenses, taxes and annual rent increases of approximately 3%. The lease will commence when the space is available for use by the Company, which is anticipated to be in the second half of 2021, and end on December 31, 2030, unless the Company earlier occupies the premises or other conditions specified in the 50 Binney Street Sublease occur. The sublessor has the right to postpone the commencement date until January 1, 2022 by providing not less than nine months' prior written notice to the Company. Upon signing the 50 Binney Street Sublease, the Company executed a $40.1 million cash-collateralized letter of credit, which may be reduced in the future subject to the terms of the 50 Binney Street Sublease and certain reduction requirements specified therein. The $40.1 million of cash collateralizing the letter of credit is classified as restricted cash and other non-current assets on the Company's condensed consolidated balance sheets. Payments will commence at the earlier of (i) the date which is 90 days following the commencement date and (ii) the date the Company takes occupancy of all or any portion of the premises. In connection with the execution of the 50 Binney Street Sublease, the Company also entered into a Purchase Agreement for furniture and equipment (the "Furniture Purchase Agreement") located on the premises upon lease commencement. Upon execution of the Furniture Purchase Agreement, the Company made an up-front payment of $7.5 million, all of which was recorded within restricted cash and other non-current assets on the Company's condensed consolidated balance sheets as of June 30, 2020. The Company will assess the lease classification of the 50 Binney Street Sublease and commence recognition of the associated rent expense upon lease commencement.

Seattle, Washington leases

In July 2018, the Company entered into a lease agreement for office and laboratory space located in a portion of a building in Seattle, Washington. The lease was amended in October 2018 to increase the total rentable space to approximately 36,126 square feet at $54.00 per square foot in base rent per year, which is subject to scheduled annual rent increases of 2.5% plus certain operating expenses and taxes. The lease commenced on January 1, 2019, and the lease term will continue through January 31, 2027. The Company moved into the facility in June 2019. The Company determined the classification of this lease to be an operating lease and recorded a right-of-use asset and lease liability at lease commencement.

In September 2019, the Company entered into a second amendment to the lease (the "Second Amendment"). The Second Amendment added approximately 22,188 square feet to the existing space and extended the lease term of the entire premises by 16 months, or until April 2028. Fixed monthly rent for the expanded space will be incurred at a rate of $62.80 per square foot per year beginning in January 2021, subject to annual increases of 2.5%. The Second Amendment includes a five-year option to extend the term.

Upon the execution of the Second Amendment, which was deemed to be a lease modification, the Company re-evaluated the assumptions made at the original lease commencement date. The Company determined the Second Amendment consists of two separate contracts under ASC 842. One contract is related to a new right-of-use for the expanded 22,188 square feet of space, which is to be accounted for as a new lease, and the other is related to the modification of term for the original 36,126 square feet of space. The Company recorded an additional right-of-use asset and lease liability upon lease commencement of the expanded space. The Company is recognizing rent expense on a straight-line basis through the remaining extended term of the respective leases.

Embedded operating leases

In June 2016, the Company entered into a manufacturing agreement for the future commercial production of the Company's beti-cel, and eli-cel drug products 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 paid $12.0 million upon the achievement of certain contractual milestones and may pay up to $8.0 million in additional contractual milestones if the Company elects its option to lease additional suites. Construction was completed in March 2018 and beginning in April 2018, the Company pays $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 at any time upon payment of a one-time termination fee and up to 24 months of fixed suite and labor fees. The Company determined 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 recorded a right-of-use asset and lease liability for this operating lease on the effective date of ASC 842 and is recognizing rent expense on a straight-line basis throughout the remaining term of the embedded lease.

In November 2016, the Company entered into an agreement for clinical and commercial production of the Company's ZYNTEGLO, LentiGlobin for SCD, and eli-cel drug products with a contract manufacturing organization at an existing facility. The Company concluded that this agreement contains an embedded operating lease as the clean rooms are designated for the Company's exclusive use during the term of the agreement. The term of the agreement is five years with subsequent three-year renewals at the mutual option of each party. As a result, the Company recorded a right-of-use asset and lease liability for this operating lease on the effective date of ASC 842, and is recognizing rent expense on a straight-line basis throughout the estimated remaining term of the embedded lease. In March 2020, the Company amended its agreement with the contract manufacturing organization, resulting in a lease modification. Under the terms of the amended arrangement, the Company may be required to pay annual maintenance and production fees of up to €16.5 million, depending on its production needs, and may terminate this agreement with twelve months' notice and a one-time termination fee. The amendment also provides for an option to reserve an additional clean room for a one-time option fee plus annual maintenance fees. As a result, the Company increased the right-of-use asset and lease liability related to this embedded operating lease during the first quarter of 2020.

8. Commitments and contingencies

Contingent consideration related to business combinations

In June 2014, the Company acquired Pregenen. The Company may be required to make up to $120.0 million in remaining future contingent cash payments to the former equityholders of Pregenen upon the achievement of certain clinical and commercial milestones related to the Pregenen technology, of which $20.1 million relates to clinical milestones and $99.9 million relates to commercial milestones. In accordance with accounting guidance for business combinations, contingent consideration liabilities are required to be recognized on the consolidated balance sheets at fair value. Estimating the fair value of contingent consideration requires the use of significant assumptions primarily relating to probabilities of successful achievement of certain clinical and commercial milestones, the expected timing in which these milestones will be achieved, and discount rates. The use of different assumptions could result in materially different estimates of fair value. Please refer to Note 4, *Fair value measurements*, for additional information.

Other funding commitments

The Company is party to various agreements, principally relating to licensed technology, that require future payments relating to milestones that may be met in subsequent periods or royalties on future sales of specified products, which includes the collaboration agreement entered into with Regeneron Pharmaceuticals, Inc. ("Regeneron") in August 2018. Please refer to Note 9, *Collaborative arrangements*, for further information on the collaboration agreement with Regeneron.

Additionally, the Company is party to various contracts with contract research organizations and contract manufacturers that generally provide for termination on notice, with the exact amounts in the event of termination to be based on the timing of the termination and the terms of the agreement. As compared to the contractual obligations and commitments as disclosed in the Company's Annual Report on Form 10-K filed with the SEC on February 18, 2020, the Company's future minimum purchase commitments as of the period ended June 30, 2020 decreased by $89.2 million primarily related to the Company's assignment of a contract manufacturing agreement to BMS; refer to Note 9, *Collaborative arrangements*, for discussion of the May 2020 amendments to the BMS arrangement for further discussion.

The Company may be obligated to make future development, regulatory, and commercial milestone payments, and royalty payments on future sales of specified products associated with its collaboration and license agreements. Payments under these

agreements generally become due and payable upon achievement of such milestones or sales. When the achievement of these milestones or sales have occurred, the corresponding amounts are recognized in the Company's financial statements. As further discussed in Note 9, *Collaborative arrangements*, BMS assumed responsibility for amounts due to licensors as a result of any future ex-U.S. sales of ide-cel and bb21217.

While there are no material legal proceedings the Company is aware of, the Company may become party to various claims and complaints arising in the ordinary course of business. 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. 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. Management does not believe that any ultimate liability resulting from any of these claims will have a material adverse effect on its results of operations, financial position, or liquidity. However, management cannot give any assurance regarding the ultimate outcome of any claims, and their resolution could be material to operating results for any particular period.

The Company also indemnifies each of its directors and officers for certain events or occurrences, subject to certain limits, while the officer or director is or was serving at the Company's request in such capacity, as permitted under Delaware law and in accordance with its certificate of incorporation and by-laws. The term of the indemnification period lasts as long as a director may be subject to any proceeding arising out of acts or omissions of such director or officer in such capacity. The maximum amount of potential future indemnification is unlimited; however, the Company currently holds director and officer liability insurance. This insurance allows the transfer of risk associated with the Company's exposure and may enable it to recover a portion of any future amounts paid. The Company believes that the fair value of these indemnification obligations is minimal. Accordingly, it has not recognized any liabilities relating to these obligations.

9. Collaborative arrangements

To date, the Company's revenue has been primarily generated from its collaboration arrangements with BMS, formerly Celgene prior to its acquisition by BMS in November 2019, and Regeneron Pharmaceuticals, Inc. ("Regeneron"), each as further described below.

Bristol-Myers Squibb

BMS Original Collaboration Agreement

In March 2013, the Company entered into a Master Collaboration Agreement (the "BMS Collaboration Agreement") with Celgene (now BMS following its acquisition of Celgene in November 2019) 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, in March 2013, the Company entered into a Platform Technology Sublicense Agreement (the "Sublicense Agreement") with BMS pursuant to which the Company obtained a sublicense to certain intellectual property from BMS, originating under BMS's license from Baylor College of Medicine, for use in the collaboration.

Under the terms of the BMS Collaboration Agreement, the Company received an up-front, non-refundable, non-creditable payment of $75.0 million. The Company was responsible for conducting discovery, research and development activities through completion of phase 1 clinical studies, if any, during the initial term of the BMS Collaboration Agreement, or three years.

BMS Amended Collaboration Agreement

In June 2015, the Company and BMS amended and restated the BMS Collaboration Agreement (the "Amended BMS Collaboration Agreement"). Under the Amended BMS Collaboration Agreement, the parties narrowed the focus of the collaboration to exclusively work on anti- B-cell maturation antigen ("BCMA") product candidates for a new three-year term. In connection with the Amended BMS Collaboration Agreement, the Company received an up-front, non-refundable, non-creditable payment of $25.0 million to fund research and development under the collaboration. Under the terms of the Amended BMS Collaboration Agreement, for up to two product candidates selected for development under the collaboration, the Company was responsible for conducting and funding all research and development activities performed up through completion of the initial phase 1 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 1 clinical study for such product candidate, the Company had granted BMS an option to obtain an exclusive worldwide license to develop and commercialize such product. Following BMS's license of each product candidate, the Company is entitled to elect to co-develop and co-promote each product candidate in the U.S.

BMS Ide-cel License Agreement

In February 2016, BMS exercised its option to obtain an exclusive worldwide license to develop and commercialize ide-cel, the first product candidate under the Amended BMS Collaboration Agreement, pursuant to an executed license agreement ("Ide-cel License Agreement") entered into by the parties in February 2016 and paid to the Company the associated $10.0 million option fee. Pursuant to the Ide-cel License Agreement, BMS was responsible for development and related funding of ide-cel after the substantial completion of the phase 1 clinical trial. The Company was responsible for the manufacture of vector and associated payload throughout development and upon BMS's request, throughout commercialization, the costs of which were reimbursable by BMS in accordance with the terms of the Amended and Restated Co-Development, Co-Promote and Profit Share Agreement, as further described below. BMS was responsible for the manufacture of drug product throughout development and commercialization. Under the Ide-cel License Agreement, the Company was eligible to receive U.S. milestones of up to $85.0 million for the first indication to be addressed by ide-cel and royalties for U.S. sales of ide-cel. Additionally, the Company was eligible to receive ex-U.S. milestones of up to $55.0 million and royalties for ex-U.S. sales of ide-cel.

BMS Ide-cel Co-Development, Co-Promote and Profit Share Agreement

In March 2018, the Company elected to co-develop and co-promote ide-cel within the U.S. pursuant to the execution of the Amended and Restated Co-Development, Co-Promote and Profit Share Agreement ("Ide-cel CCPS"), which replaced the Ide-cel License Agreement. As a result of executing the Ide-cel CCPS, the responsibilities of the parties remained unchanged from those under the Ide-cel License Agreement, however, the Company will share equally in all profits and losses relating to developing, commercializing and manufacturing ide-cel within the U.S. and has the right to participate in the development and promotion of ide-cel in the U.S. BMS is responsible for the costs incurred to manufacture vector and associated payload for use outside of the U.S., plus a mark-up. As a result of electing to co-develop and co-promote ide-cel within the U.S., the milestones and royalties payable under the Ide-cel License Agreement were adjusted. Under the Ide-cel CCPS, the Company was eligible to receive a $10.0 million milestone related to the development of ide-cel in the U.S. and, for the first indication to be addressed by ide-cel, ex-U.S. regulatory and commercial milestones of up to $60.0 million. Additionally, the Company was eligible to receive royalties for ex-U.S. sales of ide-cel, but not for U.S. sales of ide-cel. Under the Ide-cel CCPS, the $10.0 million development milestone was achieved in the second quarter of 2019 and subsequently paid by BMS.

In May 2020, the First Amendment to the Amended and Restated Co-Development, Co-Promote and Profit Share Agreement (as amended, the "Amended Ide-cel CCPS") was executed, which amended the Ide-cel CCPS. Under the Amended Ide-cel CCPS, the parties will continue to share equally in all profits and losses relating to developing, commercializing and manufacturing ide-cel within the U.S. Under the Amended Ide-cel CCPS and the Amended bb21217 License Agreement, described further below, BMS was relieved of its obligations to pay the Company for future ex-U.S. milestones and royalties on ex-U.S. sales for each of ide-cel and bb21217 in exchange for an up-front, non-refundable, non-creditable payment of $200.0 million, which represents the aggregate of the probability-weighted, net present value of the future ex-U.S. milestones and royalties on ex-U.S. sales for each of ide-cel and bb21217. In connection with these amendments, BMS assumed a contract manufacturing agreement relating to ide-cel lentiviral vector. Over time, BMS will assume responsibility for manufacturing ide-cel lentiviral vector for use outside of the U.S., with bluebird retaining responsibility for manufacturing ide-cel lentiviral vector for use within the U.S. during development and, upon request, throughout commercialization. In addition, under the Amended Ide-cel CCPS and the Amended bb21217 License Agreement, described further below, the parties are released from future exclusivity related to BCMA-directed T cell therapies. There are no remaining milestones or royalties under the Amended Ide-cel CCPS.

BMS bb21217 License Agreement

In September 2017, BMS exercised its option to obtain an exclusive worldwide license to develop and commercialize bb21217, the second product candidate under the Amended BMS Collaboration Agreement, pursuant to an executed license agreement ("bb21217 License Agreement") entered into by the parties in September 2017 and paid the Company an option fee of $15.0 million. Pursuant to the bb21217 License Agreement, BMS is responsible for development and related funding of bb21217 after the substantial completion of the ongoing phase 1 clinical trial. In 2019, the parties amended the protocol for the ongoing phase 1 clinical trial to enroll additional patients for which the Company will be reimbursed based upon an agreed-upon amount per patient. Under the bb21217 License Agreement, the Company is eligible to receive U.S. milestones of up to

$85.0 million for the first indication to be addressed by bb21217 and royalties for U.S. sales of bb21217. Additionally, the Company was eligible to receive ex-U.S. milestones of up to $55.0 million and royalties for ex-U.S. sales of bb21217.

In May 2020, the Second Amended and Restated License Agreement ("Amended bb21217 License Agreement") was executed, which replaced the bb21217 License Agreement. Under the Amended bb21217 License Agreement, over time, BMS will assume responsibility for manufacturing bb21217 lentiviral vector for use outside of the U.S., with bluebird retaining responsibility for manufacturing bb21217 lentiviral vector for use within the U.S. during development and, upon request, throughout commercialization. Under the Amended bb21217 License Agreement, expenses incurred by the Company associated with these activities are fully reimbursable by BMS at cost plus a mark-up. Throughout both development and commercialization, BMS is responsible for the manufacture of drug product. There are no remaining milestones and royalties related to the ex-U.S. development or commercialization of bb21217 following execution of the Amended bb21217 License Agreement.

The Company currently expects it will exercise its option to co-develop and co-promote bb21217 within the U.S. The Company's election to co-develop and co-promote bb21217 must be made by the substantial completion of the on-going phase 1 clinical trial of bb21217. If elected, the Company expects the responsibilities of the parties to remain largely unchanged, however, the Company expects it will share equally in all profits and losses relating to developing, commercializing and manufacturing bb21217 within the U.S. and to have the right to participate in the development and promotion of bb21217 in the U.S. Under this scenario, the U.S. milestones and royalties payable under the bb21217 License Agreement would be adjusted and the Company would be eligible to receive a $10.0 million development milestone payment related to the development of bb21217 within the U.S. The Company would not be eligible for royalties on U.S. sales of bb21217 under this scenario.

In the event the Company does not exercise its option to co-develop and co-promote bb21217, the Company will receive an additional fee in the amount of $10.0 million. Under this scenario, there would be no change to the U.S. milestones and royalties for U.S. sales of bb21217, as previously described above, for which the Company would be eligible to receive.

Accounting Analysis – Amended Ide-cel CCPS and Amended bb21217 License Agreement

In accordance with the Company's accounting policies related to variable consideration, as further described in the Company's Annual Report on Form 10-K filed with the SEC on February 18, 2020, if an arrangement includes variable consideration, including milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price of an arrangement. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's control, such as regulatory approvals, are generally not considered probable of being achieved until those approvals are received. The Company recognizes royalty revenue and sales-based milestones at the later of (i) when the related sales occur, or (ii) when the performance obligation to which the royalty has been allocated has been satisfied.

Prior to the May 2020 amendments, the Company had constrained all variable consideration related to the remaining ex-U.S. milestones and royalties for ex-U.S. sales under the Ide-cel CCPS and bb21217 License Agreement. As a result of the May 2020 amendments, the uncertainty associated with the previously constrained variable consideration for future ex-U.S. milestones and royalties on ex-U.S. sales for each of ide-cel and bb21217 was resolved in exchange for an up-front, non-refundable, non-creditable payment of $200.0 million.

While the Ide-cel CCPS and bb21217 License Agreement were historically accounted for as separate contracts, the May 2020 amendments to each agreement were negotiated as a package with a single commercial objective and, as such, the Amended Ide-cel CCPS and Amended bb21217 License Agreement were combined for accounting purposes and treated as a single arrangement.

At the time of the May 2020 amendments, there was one remaining performance obligation under each of the Ide-cel CCPS and bb21217 License Agreement, neither of which were fully satisfied: a combined performance obligation of the ide-cel license and ide-cel vector manufacturing through development; and a combined performance obligation of the bb21217 license and bb21217 vector manufacturing through development. Subsequent to the May 2020 amendments, the Company concluded the two performance obligations are distinct from each other as BMS can benefit from each license and associated manufacturing services separately and the respective licenses and manufacturing services do not modify one another and are not interdependent. Accordingly, the Company will continue to account for each performance obligation separately.

The Company allocated the $200.0 million up-front payment received in connection with the May 2020 amendments to the remaining performance obligations described above based on the general allocation principles of Topic 606. In applying these

principles, the Company considered the $200.0 million up-front payment is representative of previously constrained variable consideration that has been changed and the related uncertainties resolved by the May 2020 amendments. Moreover, the Company considered that a portion of the $200.0 million was specifically attributable to each remaining performance obligation as the amount represents the aggregate of the probability-weighted, net present value of the future ex-U.S. milestones and royalties on ex-U.S. sales for each of ide-cel and bb21217 and that each respective portion therefore (i) relates specifically to the Company's satisfaction of each of its remaining performance obligations and (ii) is representative of the amount of consideration the Company expects to be entitled to in exchange for satisfying the respective performance obligations. As such, the Company concluded that the portion of the $200.0 million up-front payment specifically attributable to each of ide-cel and bb21217 should be allocated to each respective performance obligation pursuant to the variable consideration allocation exception.

The Amended Ide-cel CCPS and Amended bb21217 License Agreement represent a contract modification to an existing contract under Topic 606 given the May 2020 amendments resulted in a reduction in scope of the Company's responsibilities under each performance obligation described above. Specifically, the May 2020 amendments reduced the scope of the Company's obligation to provide ex-U.S. vector manufacturing services through development for both ide-cel and bb21217 as those activities will transition to BMS over time. In addition, the May 2020 amendments resulted in a change in the overall transaction price under the arrangement. The May 2020 amendments did not include any additional promised goods and services.

The remaining goods and services to be provided in order to fully satisfy each performance obligation described above are not distinct from those previously provided with respect to each performance obligation. Therefore, for each performance obligation, the remaining goods and services are part of a single performance obligation that is partially satisfied at the date of the contract modification. Accordingly, the effect that the contract modification had on the transaction price and the measure of progress toward complete satisfaction of each respective performance obligation has been recognized on a cumulative catch-up basis. The accounting for any previously satisfied performance obligations as of the contract modification date are not affected by the modification.

Ide-cel transaction price

The following tables summarize the total transaction price, the allocation of the total transaction price to the identified performance obligations under the arrangement (including those performance obligations that were completed as of the May 2020 contract modification date), and the amount of the transaction price unsatisfied as of June 30, 2020 (in thousands):

	Ide-cel transaction price as of June 30, 2020
Up-front non-refundable payments, option fee and milestone payments received prior to May 2020 contract modification (1)	$ 120,000
Allocated portion of the up-front non-refundable payment received in connection with the Amended Ide-cel CCPS and bb21217 License Agreement (2)	184,029
Estimated variable consideration (3)	83,083
	$ 387,112

(1) Composed of all up-front payments and option fee and milestone payments received under the BMS Collaboration Agreement, Amended BMS Collaboration Agreement, Ide-cel License Agreement, and Ide-cel CCPS. This consideration was allocated to the performance obligations under the Ide-cel CCPS based on a relative standalone selling price (“SSP”) basis. The Company estimated the SSP of the ide-cel license after considering potential future cash flows under the license. The Company then discounted these probability-weighted cash flows to their present value. The Company estimated the SSP of each of the ide-cel research and development services and ide-cel manufacturing services to be provided based on the Company’s estimated cost of providing the services plus an applicable profit margin commensurate with observable market data for similar services.

(2) This represents the portion of the $200.0 million up-front payment received under the Amended Ide-cel CCPS and Amended bb21217 License Agreement which was allocated to ide-cel.

(3) Estimated variable consideration represents the estimated reimbursement from BMS for the manufacture of vectors and associated payload through development.

	Allocation of transaction price to performance obligations	Transaction price unsatisfied as of June 30, 2020
Ide-cel research and development services	$ 40,912	$ —
Ide-cel license and manufacturing services	346,200	5,151
	$ 387,112	$ 5,151

Ide-cel research and development services

The Company satisfied this performance obligation as the research and development services were performed. The Company determined that the period of performance of the research and development services was three years through projected initial phase 1 clinical study substantial completion, or through May 2018. The research and development performance obligation was satisfied prior to the May 2020 amendments and, as a result, the accounting for this previously satisfied performance obligation was not affected by the modification. The Company recognized revenue related to ide-cel research and development services of $0.0 million for the three and six months ended June 30, 2020 and $2.5 million and $2.3 million for the three and six months ended June 30, 2019, respectively.

Ide-cel license and manufacturing services

The Company accounts for its vector manufacturing services for development in the U.S. and BMS's U.S. development efforts within the scope of ASC 808, *Collaborative Arrangements* ("ASC 808") given that both parties are active participants in the activities and both parties are exposed to significant risks and rewards dependent on the commercial success of the activities. The Company recognizes revenue for its U.S. manufacturing services by analogy to Topic 606. The portion of BMS's U.S. development costs that the Company is responsible for are recognized as a reduction to its collaborative arrangement revenues, or, if in excess of such revenues in a given quarter, the excess is recorded as research and development expense.

The Company recognizes revenue associated with the combined performance obligation using the proportional performance method, as the Company will satisfy this performance obligation as the manufacturing services are performed through development. In using this method, the Company estimated its development plan for ide-cel, including expected demand from BMS, and the costs associated with the manufacture of vectors and associated payload for incorporation into ide-cel. On a quarterly basis, the Company determines the proportion of effort incurred as a percentage of total effort it expects to expend. This ratio is applied to the transaction price, which includes variable consideration, allocated to the combined performance obligation consisting of the ide-cel license and manufacturing services. Management has applied significant judgment in the process of developing its budget estimates and any changes to these estimates will be recognized in the period in which they change as a cumulative catch-up.

The following table summarizes the net collaborative arrangement revenue recognized or expense incurred for the joint ide-cel development efforts in the U.S. under ASC 808 related to the combined performance obligation for the license and vector manufacturing of ide-cel in the U.S. for the three and six months ended June 30, 2020, and 2019 (in thousands):

	For the three months ended June 30,		For the six months ended June 30,	
	2020	2019	2020	2019
ASC 808 ide-cel license and manufacturing revenue - U.S. (1)	$ 108,196	$ —	$ 108,196	$ —
ASC 808 ide-cel research and development expense - U.S. (1)	$ —	$ (1,065)	$ (5,080)	$ (4,309)

(1) As noted above, the calculation of collaborative arrangement revenue or research and development expense to be recognized for joint ide-cel development efforts in the U.S. is performed on a quarterly basis. The calculation is independent of previous activity, which may result in fluctuations between revenue and expense recognition period over period, depending on the varying extent of effort performed by each party during the period.

Revenue related to the combined unit of accounting for the ex-U.S. license and vector manufacturing services is accounted for in accordance with Topic 606. The following table summarizes the revenue recognized related to the combined unit of accounting for the ide-cel ex-U.S. license and vector manufacturing services for the three and six months ended June 30, 2020, and 2019 (in thousands):

	For the three months ended June 30,		For the six months ended June 30,	
	2020	2019	2020	2019
ASC 606 ide-cel license and manufacturing revenue - ex-U.S.	$ 73,850	$ 7,899	$ 87,820	$ 16,963

As of June 30, 2020, the aggregate amount of the transaction price allocated to the combined performance obligation, which consists of the ide-cel license and manufacturing services, that is unsatisfied, or partially unsatisfied, is $5.2 million, which the Company expects to recognize as revenue as manufacturing services are provided through the remaining development period which is estimated to be completed following the successful transition of the contract manufacturing agreement relating to ide-cel lentiviral vector. As of June 30, 2020 and December 31, 2019, the Company had $3.9 million and $8.5 million, respectively, of deferred revenue associated with the combined performance obligation consisting of the ide-cel license and manufacturing services.

bb21217 transaction price

The following tables summarize the total transaction price, the allocation of the total transaction price to the identified performance obligations under the arrangement (including those performance obligations that were completed as of the May 2020 contract modification date), and the amount of the transaction price unsatisfied as of June 30, 2020 (in thousands):

	bb21217 transaction price as of June 30, 2020
Up-front non-refundable payment received prior to May 2020 contract modification (1)	$ 15,000
Allocated portion of the up-front non-refundable payment received in connection with the Amended Ide-cel CCPS and bb21217 License Agreement (2)	15,971
Estimated variable consideration (3)	1,803
	$ 32,774

(1) Composed of the up-front non-refundable payment received under the bb21217 License Agreement. This consideration was allocated to the performance obligations under the bb21217 License Agreement based on a relative SSP basis. The Company estimated the SSP of the bb21217 license after considering potential future cash flows under the license. The Company then discounted these probability-weighted cash flows to their present value. The Company estimated the SSP of each of the bb21217 research and development services and bb21217 manufacturing services to be provided based on the Company's estimated cost of providing the services plus an applicable profit margin commensurate with observable market data for similar services.

(2) This represents the portion of the $200.0 million up-front payment received under the Amended Ide-cel CCPS and Amended bb21217 License Agreement which was allocated to bb21217.

(3) Estimated variable consideration represents the estimated reimbursement from BMS for the manufacture of vectors and associated payload through development.

	Allocation of transaction price to performance obligations	Transaction price unsatisfied as of June 30, 2020
bb21217 research and development services	$ 5,444	$ —
bb21217 license and manufacturing services	27,330	27,330
	$ 32,774	$ 27,330

All of the remaining development, regulatory, and commercial milestones under the Amended bb21217 License Agreement are related to U.S. development, regulatory and commercialization activities and are fully constrained and are therefore excluded from the transaction price. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones is outside the control of the Company and contingent upon the future success of its clinical trials, the licensee's efforts, or the receipt of regulatory approval. Any consideration related to U.S. sales-based milestones (including royalties) will be recognized when the related sales occur as these amounts have been determined to relate predominantly to the license granted to BMS and therefore are recognized at the later of when the performance obligation is satisfied, or the related sales occur.

The Company re-evaluates the transaction price, including its estimated variable consideration included in the transaction price and all constrained amounts, each reporting period and as uncertain events are resolved or other changes in circumstances occur.

bb21217 research and development services

The Company satisfied this performance obligation as the research and development services were performed. The Company determined that the period of performance of the research and development services was two years through projected substantial completion of the initial phase 1 clinical study, or through September 2019. The research and development performance obligation was satisfied prior to the May 2020 amendments, and as a result, the accounting for this previously satisfied performance obligation was not affected by the modification. As part of performing its initial obligation to complete a phase 1 trial as originally contemplated, the Company recognized revenue of $0.0 million for the three and six months ended June 30, 2020 and $0.7 million and $1.4 million for the three and six months ended June 30, 2019, respectively.

The agreement to expand the bb21217 phase 1 trial that occurred in 2019 was previously treated as a separate contract for accounting purposes, because the trial expansion was for the addition of a promised good or service that is distinct and the associated consideration reflected the standalone selling price of the additional promised good or service. This contract was not affected by the May 2020 amendments and, accordingly, the accounting for this agreement was not impacted by the May 2020 amendments. The transaction price associated with these additional patients consists of variable consideration and is based upon an agreed-upon amount per patient which will be recognized as revenue as the patients are treated. The Company began fulfilling the performance obligation in the fourth quarter of 2019 and it remained partially unsatisfied as of June 30, 2020. In connection with treating additional patients in the phase 1 trial, the Company recognized revenue of $4.0 million and $6.4 million for the three and six months ended June 30, 2020, respectively.

bb21217 license and manufacturing services

The Company will satisfy its performance obligation related to the manufacture of vectors and associated payload for incorporation into bb21217 through development as the bb21217 manufacturing services are performed. As of June 30, 2020, the manufacturing services for bb21217 had not yet commenced. Therefore, no amounts have been recognized for the combined performance obligation in the condensed consolidated statements of operations and comprehensive loss for the three and six months ended June 30, 2020, and 2019.

The aggregate amount of the transaction price allocated to the combined performance obligation, which consists of the bb21217 license and manufacturing services, is $27.3 million. The Company does not expect that recognition will begin in the next twelve months and has therefore classified deferred revenue associated with the combined performance obligation as deferred revenue, net of current portion on its consolidated balance sheets. The Company had $25.8 million and $9.8 million of remaining deferred revenue as of June 30, 2020 and December 31, 2019, respectively, associated with the combined performance obligation consisting of the bb21217 license and manufacturing services.

Contract assets and liabilities – ide-cel and bb21217

The Company receives payments from its collaborative partners based on billing schedules established in each contract. Up-front payments and fees are recorded as deferred revenue upon receipt or when due until such time as the Company satisfies its performance obligations under these arrangements. A contract asset is a conditional right to consideration in exchange for goods or services that the Company has transferred to a customer. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional.

The following table presents changes in the balances of the Company's BMS receivables and contract liabilities during the six months ended June 30, 2020 (in thousands):

	Balance at December 31, 2019	Additions	Deductions	Balance at June 30, 2020
Receivables	$ 400	$ 6,400	$ (2,800)	$ 4,000
Contract liabilities:				
Deferred revenue	$ 18,265	$ 200,000	$ (188,588)	$ 29,677

The change in the receivables balance for the six months ended June 30, 2020 is primarily driven by amounts owed to the Company for bb21217 research and development services provided in the first half of 2020 (expanded phase 1 clinical trial), offset by amounts collected from BMS in the period.

The increase in deferred revenue during the six months ended June 30, 2020 is driven by the $200.0 million consideration received in connection with the May 2020 amendments, offset by revenue recognized in the year-to-date period related to the combined unit of accounting for ide-cel license and vector manufacturing services. A total of $188.6 million was released from deferred revenue during the year-to-date period, of which $169.2 million is related to a cumulative catch-up adjustment to revenue recorded in connection with the May 2020 contract modification described further above. As of December 31, 2019, the Company had $8.5 million of deferred revenue associated with the combined performance obligation consisting of the ide-cel license and manufacturing services, of which $7.3 million was released during the six months ended June 30, 2020.

Regeneron

Regeneron Collaboration Agreement

In August 2018, the Company entered into a Collaboration Agreement (the "Regeneron Collaboration Agreement") with Regeneron pursuant to which the parties will apply their respective technology platforms to the discovery, development, and commercialization of novel immune cell therapies for cancer. In August 2018, following the completion of required regulatory reviews, the Regeneron Collaboration Agreement became effective. Under the terms of the agreement, the parties will leverage Regeneron's proprietary platform technologies for the discovery and characterization of fully human antibodies, as well as T cell receptors directed against tumor-specific proteins and peptides and the Company will contribute its field-leading expertise in gene therapy.

In accordance with the Regeneron Collaboration Agreement, the parties jointly selected six initial targets and intend to equally share the costs of research up to the point of submitting an IND application for a potential gene therapy product directed to a particular target. Additional targets may be selected to add to or replace any of the initial targets during the five-year research collaboration term as agreed to by the parties.

Regeneron will accrue a certain number of option rights exercisable against targets as the parties reach certain milestones under the terms of the agreement. Upon the acceptance of an IND for the first product candidate directed to a target, Regeneron will have the right to exercise an option for co-development/co-commercialization of product candidates directed to such target on a worldwide or applicable opt-in territory basis, with certain exceptions. Where Regeneron chooses to opt-in, the parties will share equally in the costs of development and commercialization, and will share equally in any profits or losses therefrom in applicable opt-in territories. Outside of the applicable opt-in territories, the target becomes a licensed target and Regeneron would be eligible to receive, with respect to any resulting product, milestone payments of up to $130.0 million per product and royalties on net sales outside of the applicable opt-in territories at a rate ranging from the mid-single digits to low-double digits. A target would also become a licensed target in the event Regeneron does not have an option to such target, or Regeneron does not exercise its option with respect to such target.

Either party may terminate a given research program directed to a particular target for convenience, and the other party may elect to continue such research program at its expense, receiving applicable cross-licenses. The terminating party will receive licensed product royalties and milestone payments on the potential applicable gene therapy products. Where the Company terminates a given research program for convenience, and Regeneron elects to continue such research program, the parties will enter into a transitional services agreement. Under certain conditions, following its opt-in, Regeneron may terminate a given collaboration program and the Company may elect to continue the development and commercialization of the applicable potential gene therapy products as licensed products.

Regeneron Share Purchase Agreement

A Share Purchase Agreement ("SPA") was entered into by the parties in August 2018. In August 2018, the closing date of the transaction, the Company issued Regeneron 0.4 million shares of the Company's common stock, subject to certain restrictions, for $238.10 per share, or $100.0 million in the aggregate. The purchase price represents $63.0 million worth of common stock plus a $37.0 million premium, which represents a collaboration research advancement, or credit to be applied to Regeneron's initial 50 percent funding obligation for collaboration research, after which the collaborators will continue to fund ongoing research equally. The collaboration research advancement only applies to pre-IND research activities and is not refundable or creditable against post-IND research activities for any programs where Regeneron exercises their opt-in rights.

Accounting analysis – Regeneron

At the commencement of the arrangement, two units of accounting were identified, which are the issuance of 0.4 million shares of the Company's common stock and joint research activities during the five-year research collaboration term. The Company determined the total transaction price to be $100.0 million, which comprises $54.5 million attributed to the equity sold to Regeneron and $45.5 million attributed to the joint research activities. In determining the fair value of the common stock at closing, the Company considered the closing price of the common stock on the closing date of the transaction and included a lack of marketability discount because Regeneron received shares subject to certain restrictions.

The Company analyzed the joint research activities to assess whether they fall within the scope of ASC 808, and will reassess this throughout the life of the arrangement based on changes in the roles and responsibilities of the parties. Based on the terms of the arrangement as outlined above, for the collaboration research performed prior to submission of an IND application for a potential gene therapy product, both parties are deemed to be active participants in the collaboration. Both parties are performing research and development activities and will share equally in these costs through IND. Additionally, Regeneron and the Company are exposed to significant risks and rewards dependent on the commercial success of any product candidates that may result from the collaboration. As such, the collaboration arrangement is deemed to be within the scope of ASC 808.

The $45.5 million attributed to the joint research activities includes the $37.0 million creditable against amounts owed to the Company by Regeneron. The collaboration research advancement will be reduced over time for amounts due to the Company by Regeneron as a result of the parties agreeing to share in the costs of collaboration research equally. The remainder of the amount attributed to the joint research activities will be recognized over the five-year research collaboration term.

Consistent with its collaboration accounting policy, the Company will recognize collaborative arrangement revenue or research and development expense related to the joint research activities in future periods depending on the amounts incurred by each party in a given reporting period. That is, if the Company's research costs incurred exceed those research costs incurred by Regeneron in a given quarter, the Company will record collaborative arrangement revenue and reduce the original $37.0 million advance by the amount due from Regeneron until such advancement is fully utilized, after which the Company would record an amount due from Regeneron. If Regeneron's research costs incurred exceed those research costs incurred by the Company in a given quarter, the Company will record research and development expense and record a liability for the amount due to Regeneron. As of June 30, 2020 and December 31, 2019, the Company has $34.4 million and $38.2 million, respectively, of the amount attributed to the joint research activities remaining to be recognized which is classified as collaboration research advancement, current portion and collaboration research advancement, net of current portion on the condensed consolidated balance sheets.

The Company recognized $1.5 million and $3.8 million of collaborative arrangement revenue from the Regeneron Collaboration Agreement during the three and six months ended June 30, 2020, respectively. The Company recognized $0.5 million and $2.5 million of collaborative arrangement revenue from the Regeneron Collaboration Agreement during the three and six months ended June 30, 2019, respectively.

10. Equity

In May 2020, the Company sold 10.5 million shares of common stock (inclusive of shares sold pursuant to an option granted to the underwriters in connection with the offering) through an underwritten public offering at a price of $55.00 per share for aggregate net proceeds of $541.5 million.

11. Stock-based compensation

In January 2020 and 2019, the number of shares of common stock available for issuance under the 2013 Stock Option and Incentive Plan ("2013 Plan") was increased by approximately 2.2 million and 2.2 million shares, respectively, as a result of the automatic increase provision of the 2013 Plan. As of June 30, 2020, the total number of shares of common stock available for issuance under the 2013 Plan was approximately 2.4 million.

Stock-based compensation expense

The Company recognized stock-based compensation expense totaling $48.5 million and $84.8 million for the three and six months ended June 30, 2020, respectively. The Company recognized stock-based compensation expense totaling $55.1 million and $87.5 million for the three and six months ended June 30, 2019, respectively. Stock-based compensation expense by award type included within the condensed consolidated statements of operations and comprehensive loss was as follows (in thousands):

	For the three months ended June 30,		For the six months ended June 30,	
	2020	2019	2020	2019
Stock options	$ 26,073	$ 24,800	$ 50,513	$ 47,983
Restricted stock units	14,143	30,012	25,996	38,893
Employee stock purchase plan and other	8,313	299	8,313	576
	$ 48,529	$ 55,111	$ 84,822	$ 87,452

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

	For the three months ended June 30,		For the six months ended June 30,	
	2020	2019	2020	2019
Research and development	$ 23,098	$ 29,694	$ 39,367	$ 45,210
Selling, general and administrative	25,431	25,417	45,455	42,242
	$ 48,529	$ 55,111	$ 84,822	$ 87,452

As of June 30, 2020, the Company had approximately $332.0 million of unrecognized stock-based compensation expense, which is expected to be recognized over a weighted-average period of approximately 2.5 years.

Stock option activity

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

	Shares (in thousands)	Weighted-average exercise price per share
Outstanding at December 31, 2019	5,483	$ 116.30
Granted	1,306	$ 72.50
Exercised	(27)	$ 40.91
Canceled, forfeited, or expired	(296)	$ 135.10
Outstanding at June 30, 2020	6,466	$ 106.90
Exercisable at June 30, 2020	3,442	$ 102.07
Vested and expected to vest at June 30, 2020	6,466	$ 106.90

During the six months ended June 30, 2020, less than 0.1 million stock options were exercised, resulting in total proceeds to the Company of $1.1 million.

Restricted stock unit activity

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

	Shares (in thousands)	Weighted-average grant date fair value
Unvested balance at December 31, 2019	1,127	$ 146.10
Granted	879	$ 72.59
Vested	(318)	$ 143.73
Forfeited	(95)	$ 132.84
Unvested balance at June 30, 2020	1,593	$ 106.81

Employee stock purchase plan

In June 2013, the Company adopted its 2013 Employee Stock Purchase Plan ("2013 ESPP"), which authorized the initial issuance of up to a total of 0.2 million shares of the Company's common stock to participating employees. During each of the six months ended June 30, 2020 and 2019, less than 0.1 million shares of common stock were issued under the 2013 ESPP.

12. 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. The tax expense recognized during the three and six months ended June 30, 2020 is due to income taxes on foreign earnings, offset by a deferred tax benefit for which a corresponding tax expense is recognized in other comprehensive income (loss).

In March 2020, the Coronavirus Aid, Relief and Economic Security Act ("CARES Act") was enacted. This law temporarily suspended and adjusted certain law changes enacted in the Tax Cuts and Jobs Act in 2017. The Company has concluded that the provisions in the CARES Act have an immaterial impact on the Company's income tax expense, net deferred tax assets and associated valuation allowance.

13. 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 six months ended June 30,	
	2020	2019
Outstanding stock options	6,466	5,393
Restricted stock units	1,593	1,103
ESPP shares and other	323	15
	8,382	6,511

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 18, 2020.

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 biotechnology company committed to researching, developing, and commercializing potentially transformative gene therapies for severe genetic diseases and cancer. We have built an integrated product platform with broad therapeutic potential in a variety of indications based on our lentiviral gene addition platform, gene editing and cancer immunotherapy capabilities. We believe that gene therapy for severe genetic diseases has the potential to change the way patients living with these diseases are treated by addressing the underlying genetic defect that is the cause of their disease, rather than offering treatments that only address their symptoms. Our gene therapy programs in severe genetic diseases include beti-cel, LentiGlobin for SCD gene therapy, and eli-cel. Our programs in oncology are focused on developing novel T cell-based immunotherapies, including CAR and TCR T cell therapies. bb2121 (idecabtagene vicleucel, or ide-cel), and bb21217 are CAR-T cell product candidates for the treatment of multiple myeloma and partnered under our collaboration arrangement with BMS.

We are commercializing beti-cel as ZYNTEGLO in the European Union and expect to begin to generate product revenue in the second half of 2020. We are engaged with the European Medicines Agency, or EMA, in discussions regarding our proposed development plans for beti-cel as a treatment for patients with TDT who are less than 12 years of age and for patients who have a β^0/β^0 genotype. We are engaged with the U.S. Food and Drug Administration, or FDA, in discussions regarding our proposed development plans for beti-cel as a treatment for patients with TDT. We currently expect to complete our BLA submission for beti-cel for the treatment of patients with TDT in mid-2021.

Based on our discussions with the FDA, we believe that we may be able to seek accelerated approval for LentiGlobin for SCD in the United States on the basis of clinical data from Group C of our ongoing HGB-206 clinical study, with a potential first submission in the second half of 2021, and with our ongoing HGB-210 clinical study providing confirmatory data for full approval. We are also engaged with the EMA in discussions regarding our proposed development plans for LentiGlobin for SCD in Europe. LentiGlobin for SCD has received the rare pediatric disease designation by the FDA's Office of Orphan Drugs.

Based on our discussions with the FDA and EMA, we believe that we may be able to seek approval for eli-cel for the treatment of patients with CALD on the basis of our clinical data from our ongoing Starbeam study, safety data from our

ongoing ALD-104 study, and the completed ALD-103 observational study. We expect to submit a Marketing Authorization Application in the EU for eli-cel for the treatment of patients with CALD by year-end 2020. We expect to submit the BLA for eli-cel for the treatment of patients with CALD in mid-2021.

In collaboration with BMS (which acquired Celgene in November 2019), we are developing the ide-cel and bb21217 product candidates as treatments for multiple myeloma, a hematologic malignancy that develops in the bone marrow and is fatal if untreated. We are co-developing and co-promoting ide-cel in the United States with BMS and we have exclusively licensed to BMS the development and commercialization rights for ide-cel outside of the United States. In the third quarter of 2020, BMS resubmitted the BLA for ide-cel as a treatment for relapsed and refractory multiple myeloma. This resubmission in the third quarter of 2020 provided further details to address outstanding regulatory requests received from the FDA in May 2020 following the original BLA submission in March 2020. We have exclusively licensed the development and commercialization rights for the bb21217 product candidate to BMS, with an option for us to elect to co-develop and co-promote bb21217 within the United States. In May 2020, we and BMS entered into an amendment and restatement of the ide-cel co-promotion/co-development agreement, an amendment and restatement of the bb21217 license agreement, and a non-exclusive license agreement to certain patent rights controlled by us and related to lentiviral vector technology for BMS to develop and commercialize CD19-directed CAR T cell therapies.

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 selling, general and administrative support for these operations and to protect our intellectual property. We 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.

As of June 30, 2020, we had cash, cash equivalents and marketable securities of approximately $1.60 billion. We have never been profitable and have incurred net losses in each year since inception. Our net loss was $21.5 million and $224.1 million for the three and six months ended June 30, 2020, respectively, and our accumulated deficit was $2.51 billion as of June 30, 2020. Substantially all of our net losses resulted from costs incurred in connection with our research and development programs and from selling, general and administrative costs associated with our operations. We expect to continue to incur significant expenses and 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 clinical programs in β-thalassemia, SCD, and ALD, fund our share of the costs of clinical studies for our program in multiple myeloma in collaboration with BMS, and advance our preclinical programs into clinical development;
- increase research and development-related activities for the discovery and development of product candidates in severe genetic diseases and oncology;
- manufacture clinical study materials and establish the infrastructure necessary to support and develop large-scale manufacturing capabilities;
- seek regulatory approval for our product candidates;
- add personnel to support our product development and commercialization efforts; and
- increase activities related to the commercialization of ZYNTEGLO in multiple markets in Europe, the potential commercial launch of ZYNTEGLO in the United States, and the potential commercial launches of additional late-stage product candidates in the United States and Europe.

We do not expect to generate revenue from product sales until the second half of 2020. While we are in the process of completing construction and qualification of our internal lentiviral vector manufacturing capacity, currently 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. As we seek to obtain regulatory approval for our product candidates and begin to commercialize ZYNTEGLO, we expect to incur significant commercialization expenses as we prepare for product sales, marketing, manufacturing, and distribution. Accordingly, until we generate significant revenues from product sales, 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.

Business update

Beginning in late 2019, the outbreak of a novel strain of coronavirus (COVID-19) has evolved into a global pandemic. As a result, we continue to experience disruptions and increased risk in our operations and those of third parties upon whom we rely, which may materially and adversely affect our business. These include disruptions and risks related to the conduct of our clinical trials and commercialization efforts, as policies at various clinical sites and federal, state, local and foreign laws, rules and regulations continue to evolve, including quarantines, travel restrictions, and direction of healthcare resources toward pandemic response efforts. We currently expect the COVID-19 pandemic to delay the timing of patient enrollment and treatment in our ongoing clinical studies, which vary by clinical study and by program. It is unknown how long these disruptions could continue. In addition, we expect the COVID-19 pandemic to impact our ability to achieve market access and reimbursement for ZYNTEGLO in Europe due to shifting priorities of the local authorities and healthcare system.

We continue to evaluate the impact of the COVID-19 global pandemic on patients, healthcare providers and our employees, as well as our operations and the operations of our business partners and healthcare communities. In response to the COVID-19 pandemic, since early March 2020 we have adopted policies at our locations to mitigate the risk of exposure to COVID-19 by our personnel, including restrictions on the number of staff in any given research and development laboratory or manufacturing facility, a work-from-home policy applicable to the majority of our personnel, and a phased approach to bringing personnel back to our locations over time. Given the importance of supporting our patients, we are diligently working with our suppliers, healthcare providers and partners to provide patients with access to ZYNTEGLO, while taking into account regulatory, institutional, and government guidance, policies and protocols. Further, we are working with our clinical study sites to understand the duration and scope of the impact on enrollment, develop protocols to help mitigate the impact of the COVID-19 pandemic, and other activities for our ongoing clinical studies.

Given the ongoing impact of the COVID-19 global pandemic and recent shifts in regulatory timelines, in the first half of 2020 we completed a comprehensive business review with the goal of ensuring the ability to achieve our 2022 vision with a path towards financial sustainability. Under our revised business priorities and operating plan, we remain on track for potential regulatory approval and commercial launch for beti-cel, ide-cel, eli-cel, and LentiGlobin for SCD by 2022. Through this comprehensive business review, we have prioritized our key research and development programs and have made a number of changes to our future cost structure relative to our prior long-range plan, including prioritized research and development expenses, and reduced investment in selling, general and administrative expenses. In total, these changes are expected to result in over $500.0 million of net cash savings through 2022 compared to our prior long-range plan, however the ultimate impact of the COVID-19 pandemic on our business operations is highly uncertain and subject to change and will depend on future developments which are difficult to predict.

In May 2020, the Company and BMS entered into the Amended Ide-cel CCPS and Amended bb21217 License Agreement pursuant to which BMS modified its obligations to pay us for future ex-U.S. milestones and royalties on commercial sales by making a one-time up-front payment of $200.0 million. Additionally, in May 2020, we sold 10.5 million shares of common stock (inclusive of shares sold pursuant to an option granted to the underwriters in connection with the offering) through an underwritten public offering at a price of $55.00 per share for aggregate net proceeds of $541.5 million. As a result, based on our current business plan, we expect our cash, cash equivalents, and marketable securities of $1.60 billion as of June 30, 2020, together with projected revenue generated under our collaborative arrangements and projected sales of products, to fund our operations into 2023.

Financial operations overview

Revenues

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

To date, revenue recognized under our collaborative arrangements has been primarily generated from our collaboration arrangement with BMS. The terms of the arrangement with respect to ide-cel contain multiple promised goods or services, which include at inception: (i) research and development services, (ii) a license to ide-cel, and (iii) manufacture of vectors and associated payload for incorporation into ide-cel under the license. As of September 2017, the collaboration also included the

following promised goods or services with respect to bb21217: (i) research and development services, (ii) a license to bb21217, and (iii) manufacture of vectors and associated payload for incorporation into bb21217 under the license. We entered into an agreement with BMS to co-develop and co-promote ide-cel in March 2018, which was subsequently amended in May 2020, in which both parties will share equally in U.S. costs and profits. Revenue from our collaborative arrangements is recognized as the underlying performance obligations are satisfied.

We analyze our collaboration arrangements to assess whether they are within the scope of ASC 808, *Collaborative Arrangements* (“ASC 808”) to determine whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements within the scope of ASC 808, we first determine which elements of the collaboration are deemed to be within the scope of ASC 808 and those that are more reflective of a vendor-customer relationship and therefore within the scope of ASC 606, *Revenue from Contracts with Customers* (“Topic 606” or "ASC 606"). For elements of collaboration arrangements that are accounted for pursuant to ASC 808, an appropriate recognition method is determined and applied consistently, generally by analogy to Topic 606. Amounts that are owed to collaboration partners are recognized as an offset to collaborative arrangement revenues as such amounts are incurred by the collaboration partner. Where amounts owed to a collaboration partner exceed our collaborative arrangement revenues in a quarterly period, such amounts in excess are classified as research and development expense. For those elements of the arrangement that are accounted for pursuant to Topic 606, we apply the five-step model prescribed in Topic 606.

Effective January 1, 2020, we adopted Accounting Standards Update ("ASU") No. 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606* ("ASU 2018-18") on a retrospective basis. As a result, prior periods are presented in accordance with the new standard. Prior to the adoption of ASU 2018-18, we presented all revenue recognized under our collaborative arrangements as collaboration revenue on our condensed consolidated statement of operations and comprehensive loss. However, as we recognize revenue under our collaborative arrangements both within and outside the scope of Topic 606, we have revised our presentation of revenue on our condensed consolidated statement of operations and comprehensive loss as follows: service revenue includes revenue from collaborative partners recognized within the scope of Topic 606 and collaborative arrangement revenue includes only revenue from collaborative partners recognized outside the scope of Topic 606.

Non-refundable license fees paid to us are recognized as revenue upon delivery of the license provided there are no unsatisfied performance obligations in the arrangement. License revenue has historically been generated from out-license agreements, under which we may also recognize revenue from potential future milestone payments and royalties.

For arrangements with licenses of intellectual property that include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which the royalty has been allocated has been satisfied.

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 inventory;
- reimbursable costs to our partners for collaborative activities;
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, information technology, insurance, and other supplies in support of research and development activities;
- costs associated with our research platform and preclinical activities;
- milestones and up-front license payments;
- 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 not succeed in achieving regulatory approval for all 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, any of which could mean a significant change in the costs and timing associated with the development of our product candidates 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;
- new manufacturing processes or protocols that we may choose to or be required to implement in the manufacture of our lentiviral vector or drug product;
- regulatory feedback on requirements for regulatory approval, as well as changing standards for regulatory approval; and
- the timing and receipt of any regulatory approvals.

We plan to continue to invest in research and development for the foreseeable future as we continue to advance the development of beti-cel, eli-cel, LentiGlobin for SCD, and bb21217 product candidates, conduct research and development activities in severe genetic diseases and oncology, fund our share of the costs of development of ide-cel in collaboration with BMS, and continue the research and development of product candidates using our gene editing technology platform. Our research and development expenses include expenses associated with the following activities:

- Northstar-2 Study (HGB-207) – a multi-site, international phase 3 study to examine the safety and efficacy of beti-cel in the treatment of patients with TDT and a non-β^0/β^0 genotype.
- Northstar-3 Study (HGB-212) – a multi-site, international phase 3 study to examine the safety and efficacy of beti-cel in the treatment of patients with TDT and a β^0/β^0 genotype or an IVS-I-110 mutation.
- HGB-206 study – a multi-site phase 1/2 study in the United States to study the safety and efficacy of LentiGlobin in the treatment of patients with SCD.
- HGB-210 study – our multi-site, international phase 3 study of LentiGlobin in patients with SCD and a history of vaso-occlusive events.
- Starbeam Study (ALD-102) – a multi-site, international phase 2/3 study to examine the safety and efficacy of eli-cel in the treatment of patients with CALD.
- ALD-104 study – our multi-site, international phase 3 study to examine the safety and efficacy of eli-cel after myeloablative conditioning using busulfan and fludarabine in the treatment of patients with CALD.
- CRB-401 study – an open label, single-arm, multi-center, phase 1 study to examine the safety and efficacy of ide-cel in the treatment of patients with relapsed and refractory multiple myeloma.
- KarMMA study – an open label, single-arm, multi-center phase 2 study to examine the efficacy and safety of ide-cel in the treatment of patients with relapsed and refractory multiple myeloma.
- KarMMa-2 – a multi-cohort, open-label, multicenter phase 2 study to examine the safety and efficacy of ide-cel in the treatment of patients with relapsed and refractory multiple myeloma and in high-risk multiple myeloma
- KarMMa-3 – a multicenter, randomized, open-label phase 3 study comparing the efficacy and safety of ide-cel versus standard triplet regimens in patients with relapsed and refractory multiple myeloma.
- KarMMa-4 –, a multi-cohort, open-label, multicenter phase 1 study intended to determine the optimal target dose and safety of ide-cel in subjects with newly-diagnosed multiple myeloma
- CRB-402 study – an open label, single-arm, multicenter, phase 1 study to examine the safety and efficacy of the bb21217 product candidate in the treatment of patients with relapsed and refractory multiple myeloma.
- We will continue to incur costs related to the manufacture of clinical study materials in support of our clinical studies.

Under our revised operating plan, we have prioritized investment in research and development expenses, including an indefinite pause of the planned HGB-211 clinical study of LentiGlobin for SCD in the treatment of patients with SCD and an elevated stroke risk. We also expect that the timing of investment in our ongoing clinical studies will reflect COVID-19 related delays in enrollment and patient treatment in our HGB-206 and HGB-210 clinical studies. In addition, we have reduced or eliminated investment in certain preclinical programs, including certain academic collaborations in early pipeline activities, and implemented other cost-reduction measures.

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, 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 other research and development expenses in the table below:

	For the three months ended June 30,		For the six months ended June 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
LentiGlobin (including beti-cel)[1]	$ 30,873	$ 28,291	$ 67,754	$ 60,136
eli-cel	15,219	12,011	23,032	20,697
ide-cel	21,079	24,406	52,241	44,197
bb21217	6,955	6,031	13,026	10,517
Preclinical programs	10,696	9,871	28,146	21,200
Total direct research and development expense	84,822	80,610	184,199	156,747
Employee-and contractor-related expenses	17,906	12,203	33,810	22,721
Stock-based compensation expense	23,098	29,694	39,367	45,210
Platform-related expenses	11,492	6,209	16,509	10,836
Facility expenses	17,801	16,458	34,553	31,077
Other expenses	1,189	1,366	1,993	2,589
Total other research and development expenses	71,486	65,930	126,232	112,433
Total research and development expense	$ 156,308	$ 146,540	$ 310,431	$ 269,180

(1) Following our receipt of conditional approval for the marketing authorization of ZYNTEGLO by the European Commission in June 2019, all manufacturing costs associated with the production of LentiGlobin produced for use in the commercial sale of ZYNTEGLO in the European Union will be evaluated for capitalization as inventory on our condensed consolidated balance sheets.

Selling, general and administrative expenses

Selling, 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, legal, business development, commercial, information technology, and human resource functions. Other selling, general and administrative expenses include facility-related costs, professional fees for accounting, tax, legal and consulting services, directors' fees and expenses associated with obtaining and maintaining patents.

Under our revised operating plan, we have reduced projected selling, general and administrative expenses through both the elimination and deferral of certain costs relative to our prior long-range plan. This includes a deferred investment in building a commercial organization in the United States, reduced facilities and IT infrastructure and related costs for personnel based on our expectations for the timing of regulatory approvals and potential launch of our product candidates. However, we anticipate that our selling, general and administrative expenses, including payroll and sales and marketing expenses, will continue to increase in the future relative to current levels as we execute on our commercial launch plans in Europe for ZYNTEGLO, and perform commercial readiness activities in the United States for our product candidates.

Cost of royalty and other revenue

Cost of royalty and other revenue represents expense associated with amounts owed to third party licensors as a result of revenue recognized under our out-license arrangements.

We anticipate that our cost of royalty and other revenue will increase in the future, contingent upon the achievement of regulatory milestones. Additionally, we anticipate that our cost of royalty and other revenue will increase in the future as we expect to continue to recognize royalty revenue related to Novartis' commercial sale of tisagenlecleucel.

Change in fair value of contingent consideration

In June 2014, we acquired Precision Genome Engineering, Inc., or Pregenen. 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.

As of June 30, 2020, there are $120.0 million in future contingent cash payments, of which $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 $3.2 million as of June 30, 2020, which are classified within accrued expenses and other current liabilities and other non-current liabilities on our condensed consolidated balance sheets.

Interest income, net

For the three and six months ended June 30, 2020 and 2019, interest income, net consists primarily of interest income earned on investments.

Other income (expense), net

Other income (expense), net consists primarily of gains and losses on equity securities held by us, gains and losses on disposal of assets, and gains and losses on foreign currency.

Critical accounting policies and estimates

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 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. 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. During the six months ended June 30, 2020, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2019, which was filed with the SEC on February 18, 2020, except as otherwise described in Note 2, *Basis of presentation, principles of consolidation and significant accounting policies*, in the Notes to Condensed Consolidated Financial Statements.

Results of Operations

Comparison of the three months ended June 30, 2020 and 2019:

	For the three months ended June 30,		
	2020	2019	Change
	(in thousands)		
Revenue:			
Service revenue	$ 78,357	$ 11,093	$ 67,264
Collaborative arrangement revenue	109,674	465	109,209
Royalty and other revenue	10,859	1,738	9,121
Total revenues	198,890	13,296	185,594
Operating expenses:			
Research and development	156,308	146,540	9,768
Selling, general and administrative	68,628	68,631	(3)
Cost of royalty and other revenue	1,554	613	941
Change in fair value of contingent consideration	(1,655)	214	(1,869)
Total operating expenses	224,835	215,998	8,837
Loss from operations	(25,945)	(202,702)	176,757
Interest income, net	2,939	9,387	(6,448)
Other income (expense), net	1,551	(2,936)	4,487
Loss before income taxes	(21,455)	(196,251)	174,796
Income tax (expense) benefit	(10)	469	(479)
Net loss	$ (21,465)	$ (195,782)	$ 174,317

Revenues. Total revenue was $198.9 million for the three months ended June 30, 2020, compared to $13.3 million for the three months ended June 30, 2019. The increase of $185.6 million was primarily attributable to a cumulative catch-up adjustment to revenue recorded in connection with the May 2020 BMS contract modification, as well as an increase in royalty and other revenue primarily attributable to revenue recognized under an out-license agreement to Juno Therapeutics, Inc.

Research and development expenses. Research and development expenses were $156.3 million for the three months ended June 30, 2020, compared to $146.5 million for the three months ended June 30, 2019. The overall increase of $9.8 million was primarily attributable to the following:

- $7.8 million of increased license and milestone fees;
- $2.0 million of increased clinical trial and medical research costs; and
- $1.7 million of increased material production and other platform costs.

The increased costs were partially offset by $2.2 million of decreased laboratory expenses and $1.6 million of decreased employee compensation, benefit, and other headcount related expenses. The decrease in employee compensation includes $6.5 million of decreased stock-based compensation expense due to the recognition of expense on performance-based restricted stock units that vested in June 2019.

Selling, general and administrative expenses. Selling, general and administrative expenses were $68.6 million for both the three months ended June 30, 2020 and 2019, resulting in an overall decrease of less than $0.1 million period over period. The decrease was primarily attributable to a $3.4 million decrease in costs related to commercial readiness activities, offset by $3.3 million of increased information technology and facility-related costs.

Change in fair value of contingent consideration. The change in fair value of contingent consideration was primarily due to the change in significant unobservable inputs used in the fair value measurement of contingent consideration, including the probabilities of successful achievement of clinical and commercial milestones and discount rates.

Interest income, net. The decrease in interest income, net was primarily related to decreased interest income earned on investments.

Other income (expense), net. The change in other income (expense), net was primarily related to changes in fair value of equity securities.

Comparison of the six months ended June 30, 2020 and 2019:

	For the six months ended June 30,		
	2020	2019	Change
	(in thousands)		
Revenue:			
Service revenue	$ 95,190	$ 20,304	$ 74,886
Collaborative arrangement revenue	111,976	2,431	109,545
Royalty and other revenue	13,587	3,032	10,555
Total revenues	220,753	25,767	194,986
Operating expenses:			
Research and development	310,431	269,180	41,251
Selling, general and administrative	141,876	128,910	12,966
Cost of royalty and other revenue	2,579	1,043	1,536
Change in fair value of contingent consideration	(4,763)	510	(5,273)
Total operating expenses	450,123	399,643	50,480
Loss from operations	(229,370)	(373,876)	144,506
Interest income, net	8,294	19,489	(11,195)
Other expense, net	(2,896)	(6,325)	3,429
Loss before income taxes	(223,972)	(360,712)	136,740
Income tax (expense) benefit	(104)	484	(588)
Net loss	$ (224,076)	$ (360,228)	$ 136,152

Revenues. Total revenue was $220.8 million for the six months ended June 30, 2020, compared to $25.8 million for the six months ended June 30, 2019. The increase of $195.0 million was primarily attributable to a cumulative catch-up adjustment to revenue recorded in connection with the May 2020 BMS contract modification, as well as an increase in royalty and other revenue primarily attributable to revenue recognized under an out-license agreement to Juno Therapeutics, Inc.

Research and development expenses. Research and development expenses were $310.4 million for the six months ended June 30, 2020, compared to $269.2 million for the six months ended June 30, 2019. The overall increase of $41.3 million was primarily attributable to the following:

- $13.3 million of increased employee compensation, benefit, and other headcount related expenses, which is primarily driven by an increase in headcount to support overall growth and includes a $5.7 million decrease in stock-based compensation expense due to the recognition of expense on performance-based restricted stock units that vested in June 2019;
- $7.9 million of increased material production and other platform costs;
- $7.7 million of increased license and milestone fees;
- $4.5 million of increased clinical trial costs;
- $3.7 million of increased consulting fees; and
- $2.1 million of increased collaboration research funding costs.

Selling, general and administrative expenses. Selling, general and administrative expenses were $141.9 million for the six months ended June 30, 2020, compared to $128.9 million for the six months ended June 30, 2019. The increase of $13.0 million was primarily attributable to the following:

- $14.2 million of increased employee compensation, benefit, and other headcount related expenses, which is primarily driven by an increase in headcount to support overall growth, including an increase of $3.2 million in stock-based compensation expense; and

- $5.3 million of increased information technology and facility-related costs.

The increased costs were partially offset by $4.1 million of decreased consulting fees and $2.1 million of decreased costs related to commercial readiness activities.

Change in fair value of contingent consideration. The change in fair value of contingent consideration was primarily due to the change in significant unobservable inputs used in the fair value measurement of contingent consideration, including the probabilities of successful achievement of clinical and commercial milestones and discount rates.

Interest income, net. The decrease in interest income, net was primarily related to decreased interest income earned on investments.

Other expense, net. The decrease in other expense, net was primarily related to changes in fair value of equity securities.

Liquidity and Capital Resources

As of June 30, 2020, we had cash, cash equivalents and marketable securities of approximately $1.60 billion. Based on our current business plan, we expect our cash, cash equivalents, and marketable securities as of June 30, 2020, together with projected revenue generated under our collaborative arrangements and projected sales of products, to fund our operations into 2023. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. As of June 30, 2020, our funds are primarily held in U.S. Treasury securities, U.S. government agency securities, equity securities, corporate bonds, commercial paper and money market accounts.

We have incurred losses and cumulative negative cash flows from operations since our inception in April 1992, and as of June 30, 2020 we had an accumulated deficit of $2.51 billion. We expect that our research and development and selling, 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 public or private equity or debt financings, strategic collaborations, or other sources. The likelihood of our long-term success must be considered in light of the expenses, difficulties, and potential delays to be encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace and the complex regulatory environment in which we operate. We may never achieve significant revenue or profitable operations.

Sources of Liquidity

Cash Flows

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

	For the six months ended June 30,	
	2020	**2019**
	(in thousands)	
Net cash used in operating activities	$ (166,378)	$ (292,027)
Net cash provided by investing activities	493,854	195,513
Net cash provided by financing activities	544,085	15,004
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 871,561	$ (81,510)

Cash Flows from Operating Activities. The $125.6 million decrease in cash used in operating activities for the six months ended June 30, 2020 compared to the six months ended June 30, 2019 was primarily due to the decrease in net loss during this period of $136.2 million, which was driven by a cumulative catch-up adjustment to revenue recorded in connection with the May 2020 BMS contract modification. Cash used in operating activities was also driven by changes in operating assets and liabilities.

Cash Flows from Investing Activities. The $298.3 million increase in cash provided by investing activities for the six months ended June 30, 2020 was primarily due to a decrease in cash used to purchase marketable securities of $369.9 million, a decrease of $22.4 million in cash used to purchase property, plant and equipment, primarily related to the facility in Durham, North Carolina, and cash provided from the sales of marketable securities of $29.9 million, partially offset by a decrease of $123.9 million in proceeds received from the maturity of marketable securities, compared to the six months ended June 30, 2019.

Cash Flows from Financing Activities. The $529.1 million increase in cash provided by financing activities was driven by an increase in proceeds from public offering of common stock, net of issuance costs, in the six months ended June 30, 2020 compared to the six months ended June 30, 2019.

Contractual Obligations and Commitments

Except as discussed in Note 7, *Leases*, and Note 8, *Commitments and contingencies*, in the Notes to Condensed Consolidated Financial Statements, there have been no material changes to our contractual obligations and commitments as included in our Annual Report on Form 10-K, which was filed with the SEC on February 18, 2020.

Off-Balance Sheet Arrangements

As of June 30, 2020, we did not have any off-balance sheet arrangements as defined in the rules and regulations of the SEC.

Item 3. Quantitative and Qualitative Disclosures About Market Risks

We are exposed to market risk related to changes in interest rates. As of June 30, 2020 and December 31, 2019, we had cash, cash equivalents and marketable securities of $1.60 billion and $1.24 billion, respectively, primarily invested in U.S. government agency securities and Treasuries, equity securities, corporate bonds, commercial paper and money market accounts invested in U.S. government agency 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. If market interest rates were to increase immediately and uniformly by 100 basis points, or one percentage point, from levels at June 30, 2020, the net fair value of our interest-sensitive marketable securities would have resulted in a hypothetical decline of approximately $1.5 million.

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 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 June 30, 2020, 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 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 June 30, 2020, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

During the quarter ended June 30, 2020 there were 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, employment and other matters. While the outcome of these proceedings and claims cannot be predicted with certainty, as of June 30, 2020, 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. 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 executive 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.

Those risk factors below denoted with a "" are newly added or have been materially updated from our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or the SEC, on February 18, 2020.*

Our business may be materially and adversely affected by the ongoing COVID-19 pandemic. The COVID-19 pandemic has had, and will likely continue to have, an impact on various aspects of our business and that of third parties on which we rely. The extent to which the COVID-19 pandemic impacts our business will depend in part on future developments, which are uncertain and unpredictable in nature.

In December 2019, a novel strain of coronavirus (COVID-19) was reported and in March 2020, the World Health Organization characterized COVID-19 as a pandemic. The COVID-19 pandemic, which has continued to spread, and the related adverse public health developments, including orders to shelter-in-place, travel restrictions, and mandated business closures, have adversely affected workforces, organizations, healthcare communities, economies, and financial markets globally, leading to an economic downturn and increased market volatility. It has also disrupted the normal operations of businesses across industries, including ours. As a result of the COVID-19 pandemic, we are experiencing disruptions in our operations and business, and those of third parties upon whom we rely. We are also experiencing disruptions to the conduct of our clinical trials and commercialization efforts. We cannot reasonably assess or predict at this time the full extent of the negative impact that the COVID-19 pandemic and related effects may have on our business, financial condition, results of operations and cash flows. We expect to continue experiencing these disruptions in our operations and those of our third parties for an unknown period of time, as the trajectory of the COVID-19 pandemic remains uncertain and continues to evolve in the United States and globally. These impacts, which may materially and adversely affect our business, include the following:

- We are conducting a number of clinical studies across our programs in geographies which are affected by the COVID-19 pandemic. The COVID-19 pandemic has had, and will likely continue to have, an impact on various aspects of our clinical studies. Policies at various clinical sites and federal, state, local and foreign laws, rules and regulations are continuing to evolve, including through the implementation of quarantines and travel restrictions, and direction of healthcare resources toward pandemic response efforts. For instance, the availability of intensive care unit beds and related healthcare resources available to support activities unrelated to COVID-19 response have fluctuated with the incidence of severe cases of COVID-19 in the surrounding communities, and we anticipate that the availability of healthcare resources will continue to fluctuate and may become significantly constrained, with variability across geographies. As a result, we expect the COVID-19 pandemic to delay the timing of patient enrollment and treatment in our ongoing clinical studies, in particular in our ongoing HGB-206 and HGB-210 clinical studies. It is unknown how long these disruptions could continue. Moreover, we are commercializing ZYNTEGLO in Europe, and our ability to generate meaningful product revenue may be delayed. In addition to the constraints on healthcare systems and resources described above, which are also applicable in the commercial treatment context, we may experience decreased patient demand for our approved product because potential patients may choose not to undergo treatment, or to delay treatment, with ZYNTEGLO.
- We currently rely on third parties to manufacture, perform quality testing, and ship our lentiviral vectors and drug products for our clinical studies and support commercialization efforts. If any such third party in our supply chain is

adversely impacted by restrictions resulting from the COVID-19 pandemic, such as staffing shortages, production slowdowns and disruptions in delivery systems, our supply chain will be disrupted, which could significantly limit our ability to manufacture our lentiviral vectors and drug products for our clinical studies and for commercial use, and could affect our ability to conduct our research and development and commercial operations.

- Health regulatory agencies globally may experience disruptions in their operations as a result of the COVID-19 pandemic. The FDA and comparable foreign regulatory agencies may have slower response times or lack resources to continue to monitor our clinical studies. As a result, review, inspection, and other timelines may be materially delayed for an unknown period of time. Any de-prioritization of our clinical studies or delay in regulatory review resulting from such disruptions could materially affect the development and study of our product candidates. In addition, we have been engaging in reimbursement discussions with governmental health programs as part of our commercial preparation activities. It is not clear to what extent shifting priorities of the local health authorities and healthcare systems due to the COVID-19 pandemic will impact our ability to achieve market access and reimbursement for ZYNTEGLO across Europe.
- We have adopted policies at our locations to mitigate the risk of exposure to COVID-19 by our personnel, including restrictions on the number of staff in any given research and development laboratory or manufacturing facility, a work-from-home policy applicable to the majority of our personnel, and a phased approach to bringing personnel back to our locations over time. Our increased reliance on personnel working from home may negatively impact productivity, or disrupt, delay, or otherwise adversely impact our business. In addition, this could increase our cyber security risk, create data accessibility concerns, and make us more susceptible to communication disruptions, any of which could adversely impact our business operations or delay necessary interactions with local and federal regulators, ethics committees, manufacturing sites, research or clinical study sites and other important agencies and contractors. Furthermore, since the onset of the COVID-19 pandemic, our employees and contractors conducting research and development activities have been limited in the activities that they may conduct, and will continue to be subject to policies restricting access to our laboratories for an extended period of time. As a result, this could delay timely completion of preclinical activities, including completing Investigational New Drug-enabling studies or our ability to select future development candidates, and initiation of additional clinical trials for our development programs.
- The trading prices for our shares of common stock and other biopharmaceutical companies have been highly volatile as a result of the economic volatility and downturn caused by the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of shares of our common stock or such sales may be on unfavorable terms. In addition, a recession, depression or other sustained adverse market event resulting from the spread of the COVID-19 pandemic will materially and adversely affect our business, the value of our common stock, and our ability to operate under our revised operating plan and execute our strategy. As described above, our business and operating plan has already been impacted by the COVID-19 pandemic, the associated governmental restrictions, and the resulting economic conditions, leading us to reduce and defer costs, adjust our priorities, timelines and expectations, and implement a revised operating plan.

The extent of the impacts described above will depend on numerous evolving factors that we may not be able to accurately predict, including:

- the duration, severity, and scope of the pandemic in the United States and globally;
- the effectiveness of governmental, business and individuals' protocols and actions that have been and continue to be taken in response to the pandemic;
- the impact of the pandemic on economic activity and actions taken in response;
- the effect on patients, healthcare providers and business partners;
- demand for our products, including as a result of reduced patient visits to healthcare providers, travel restrictions, social distancing, quarantines and other containment measures;
- uncertainty as to when we will be able to resume normal clinical study enrollment and patient treatment activities, particularly at clinical study sites located in highly impacted geographies and as a result of disruptions with our clinical study sites;
- the ability to obtain or deliver sufficient and timely supplies if the production capabilities of manufacturers and suppliers is disrupted;
- our access to the debt and equity markets on satisfactory terms, or at all;
- disruptions in regulatory oversight and actions if regulators and industry professionals are expending significant and unexpected resources addressing COVID-19; and

- any closures of our and our partners' offices, operations and facilities.

In response to the COVID-19 pandemic and the resulting uncertain economic and healthcare environment, in the first half of 2020, we revised our operating plan with the intention that it would enable us to advance our corporate strategy and pipeline during this period of uncertainty. Based on our current business plan, we expect our cash, cash equivalents, and marketable securities, together with projected revenue generated under our collaborative arrangements and projected sales of products, to fund our operations into 2023. However, the internal and external costs of executing on our revised operating plan may be higher than expected, including as a result of challenges encountered in the course of planned activities. Additionally, projected revenue generated under our collaborative arrangements and projected sales of products may be less than expected. The ultimate impact of the COVID-19 pandemic on our business operations is highly uncertain and subject to change and will depend on future developments which are difficult to predict, including the duration of the pandemic, the ultimate geographic spread of the disease, additional or modified government actions, new information that will emerge concerning the severity and impact of COVID-19 and other actions taken to contain or address its impact in the short and long term, among others. We do not yet know the full extent of potential delays or impacts on our business, our commercialization efforts, our clinical studies, our research programs, healthcare systems or the global economy, and if the ultimate impact of the COVID-19 pandemic and the resulting uncertain economic and healthcare environment is more severe than we anticipated, we may not be able to execute on our revised operating plan or on our strategy. If the duration of the COVID-19 pandemic and the associated period of business and social restrictions and economic uncertainty is longer than we anticipated, our cash, cash equivalents, and marketable securities may not be sufficient to fund the activities under our revised operating plan for the time period that we anticipated, and we may be required to further revise our operating plan. To the extent the COVID-19 pandemic adversely affects our business and financial results, it may also have the effect of heightening many of the other risks described in this "Risk Factors" section and in the "Risk Factors" section of our Annual Report on Form 10-K.

Risks related to commercialization

We have limited experience as a commercial company and the marketing and sale of ZYNTEGLO or future products may be unsuccessful or less successful than anticipated.

We are beginning to commercialize ZYNTEGLO in the European Union and we have limited experience as a commercial company. Consequently, there is limited information about our ability to overcome many of the risks and uncertainties encountered by companies commercializing products in the biopharmaceutical industry. We also have several programs in late-stage clinical development. To execute our business plan, in addition to successfully marketing and selling ZYNTEGLO and any future products, we will need to successfully:

- establish and maintain, in the geographies where we hope to treat patients, relationships with qualified treatment centers who will be treating the patients who receive ZYNTEGLO and any future products;
- obtain adequate pricing and reimbursement for ZYNTEGLO and any future products in each of the jurisdictions in which we plan to commercialize approved products;
- gain regulatory acceptance for the development and commercialization of the product candidates in our pipeline;
- develop and maintain successful strategic alliances; and
- manage our spending as costs and expenses increase due to clinical trials, marketing approvals, and commercialization, including for any extension of marketing approval of ZYNTEGLO, and for any future products.

If we are not successful in accomplishing these objectives, we may not be able to develop product candidates, commercialize ZYNTEGLO or any future products, raise capital, expand our business, or continue our operations.

The commercial success of ZYNTEGLO, and of any future products, will depend upon the degree of market acceptance by physicians, patients, third-party payers and others in the medical community.

The commercial success of ZYNTEGLO and of any future products will depend in part on the medical community, patients, and third-party or governmental payers accepting gene therapy products in general, and ZYNTEGLO and any future products in particular, as medically useful, cost-effective, and safe. ZYNTEGLO and any other products that we may bring to the market may not gain market acceptance by physicians, patients, third-party payers 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 ZYNTEGLO and of any future products 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 and any future products 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;
- the pricing of our product and of any future products;
- publicity concerning our product, any future 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 payers on the benefits of our products may require significant resources and may never be successful. Our efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors. Any of these factors may cause ZYNTEGLO, or any future products, to be unsuccessful or less successful than anticipated.

If the market opportunities for our product or any future products are smaller than we believe they are, and if we are not able to successfully identify patients and achieve significant market share, our revenues may be adversely affected and our business may suffer.

We focus our research and product development on treatments for severe genetic diseases and cancer. 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 or any future products, are based on estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower or more difficult to identify than expected. For instance, because newborn screening for CALD is limited, and it can be difficult to diagnose CALD in the absence of a genetic screen, we may have difficulty reaching patients who would benefit from treatment from eli-cel. Additionally, the potentially addressable patient population for our product and any future products may be limited or may not be amenable to treatment with our products. For instance, we received conditional marketing approval in Europe of ZYNTEGLO for the treatment of adult and adolescent patients with TDT who do not have a β^0/β^0 genotype. We do not have any assurance whether or when ZYNTEGLO may be commercially available to pediatric patients less than 12 years of age, or patients with all genotypes of TDT or types of β-thalassemia, or in markets outside of Europe.

Even if we obtain significant market share for a product within an approved indication, because the potential target populations for our product and for the product candidates in our pipeline are small, we may never achieve profitability without obtaining marketing approval for additional indications. For instance, in the field of cancer, the FDA often approves new therapies initially only for use in patients with relapsed or refractory advanced disease. We expect to initially seek approval of our T cell-based product candidates in cancer in this context. Subsequently, for those products that prove to be sufficiently beneficial, if any, we would expect to seek approval in earlier lines of treatment and potentially as a first line therapy, but there is no guarantee that our product candidates, even if approved, would be approved for earlier lines of therapy, and, prior to any such approvals, we may have to conduct additional clinical trials. For example, BMS has submitted a BLA seeking approval from the FDA for ide-cel as a treatment for relapsed and refractory multiple myeloma. BMS is conducting the KarMMa-2, KarMMa-3, and KarMMa-4 studies with the intention to generate data to support marketing approvals for earlier lines of therapy in multiple myeloma, but there is no assurance that such studies will be successful or be sufficient.

Any of these factors may negatively affect our ability to generate revenues from sales of our product and any future products and our ability to achieve and maintain profitability and, as a consequence, our business may suffer.

We rely on a complex supply chain for ZYNTEGLO and our product candidates. The manufacture and delivery of our lentiviral vector and drug products present significant challenges for us, and we may not be able to produce our vector and drug products at the quality, quantities, locations or timing needed to support commercialization and clinical programs. In addition, we may encounter challenges with engaging or coordinating with qualified treatment centers needed to support commercialization.

In order to commercialize ZYNTEGLO and any future products, we will need to develop, contract for, or otherwise arrange for the necessary manufacturing capabilities. We currently rely on third parties to manufacture the vector and the drug product in the commercial setting and for any clinical trials that we initiate. Currently, SAFC is the sole manufacturer of the lentiviral vector and apceth is the sole manufacturer of the drug product to support commercialization of ZYNTEGLO in Europe. Although we intend to eventually rely on a mix of internal and third-party manufacturers to support our commercialization efforts, we are still in the process of completing construction and qualification of our internal capacity and we have not secured commercial-scale manufacturing capacity in all of the regions where we intend to commercialize ZYNTEGLO or future products. By building our own internal manufacturing facility, we have incurred substantial expenditures and expect to incur significant additional expenditures in the future. In addition, there are many risks inherent in the construction of a new facility that could result in delays and additional costs, including the need to obtain access to necessary equipment and third-party technology, if any. Also, we have had to, and will continue to, hire and train qualified employees to staff our manufacturing facility. We may not be able to timely or successfully build out our internal capacity or negotiate binding agreements with third-party manufacturers at commercially reasonable terms. If we fail to secure adequate capacity to manufacture our drug products or lentiviral vectors used in the manufacture of our drug products, we may be unable to execute on our development plans on the timing that we expect.

The manufacture of our lentiviral vector and drug product is complex and requires significant expertise. Even with the relevant experience and expertise, manufacturers of cell therapy products often encounter difficulties in production, particularly in scaling out and validating initial production, and ensuring that the product meets required specifications. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. We cannot make any assurances that these problems will not occur in the future, or that we will be able to resolve or address problems that occur in a timely manner or with available funds. Because of the complexity of manufacturing our product and product candidates, transitioning production of either lentiviral vector or drug products to backup or second source manufacturing, or to internal manufacturing capacity, requires a lengthy technology transfer process and may require additional significant financial expenditures. Furthermore, our cost of goods development is at an early stage. The actual cost to manufacture our lentiviral vector and drug product could be greater than we expect and could materially and adversely affect the commercial viability of our product and any future products. If we or such third-party manufacturers are unable to produce the necessary quantities of lentiviral vector and our drug product, or in compliance with GMP or other pertinent regulatory requirements, and within our planned time frame and cost parameters, the development and commercialization of our product and any future products may be materially harmed. Furthermore, if we or our third-party manufacturers are unable to produce our lentiviral vectors or our drug products in quantities, in accordance with regulatory requirements, including quality requirements, or within the time frames that we need to support our development and commercialization activities, it may result in delays in our plans or increased capital expenditures.

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 and product candidates. Such suppliers may not sell these key materials to us or 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 agreements for the commercial supply for all of these key materials.

Additionally, since the HSCs and T cells used as starting material for our drug products have a limited window of stability following procurement from a patient, we must establish transduction facilities in the regions where we wish to commercialize our product and any future products. Currently, we rely on third-party contract manufacturers in the United States and Europe to produce drug product for commercialization and for our clinical studies. Since a portion of our target patient populations will be outside the United States and Europe, we will need to establish additional transduction facilities that can replicate our transduction process in order to address those patient populations. Establishment of such facilities may be financially impractical or 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.

Our commercial strategy is to engage apheresis and transplant centers in our key launch regions as qualified treatment centers for the collection of patient HSCs and infusion of the drug product once manufactured. To ensure that the qualified treatment centers are prepared to collect patient HSCs and to ship them to our transduction facilities in accordance with our specifications and regulatory requirements, we plan to train and conduct quality assessments of each center as part of engagement. These qualified treatment centers are the first and last points on our complex supply chain to reach patients in the commercial setting. We may not be able to engage qualified treatment centers in all of the regions in our commercial launch strategy, or we may encounter other challenges or delays in engaging qualified treatment centers. We may fail to manage the logistics of collecting and shipping patient material to the manufacturing site and shipping the drug product back to the patient. Logistical and shipment delays and problems caused by us, our third-party vendors, and other factors not in our control, such as weather, could prevent or delay the delivery of product to patients. If our qualified treatment centers fail to perform satisfactorily, we may suffer reputational, operational, and business harm. We are required to maintain a complex chain of identity and chain of custody with respect to patient material as it moves through the manufacturing process, from the qualified treatment center to the transduction facility, and back to the patient. Failure to maintain chain of identity and chain of custody could result in adverse patient outcomes, loss of product or regulatory action.

Although we are continuing to build out our commercial capabilities, we have no prior sales or distribution experience and limited capabilities for marketing and market access. We expect to invest significant financial and management resources to establish these capabilities and infrastructure to support commercial operations. If we are unable to establish these commercial capabilities and infrastructure or to enter into agreements with third parties to market and sell our product or any future products, we may be unable to generate sufficient revenue to sustain our business.

Although we are continuing to build out our field team as part of our first commercial launch in Europe, we have no prior sales or distribution experience and limited capabilities for marketing and market access. To successfully commercialize ZYNTEGLO and any other products that may result from our development programs, we will need to develop these capabilities and further expand our infrastructure to support commercial operations in the United States, Europe and other regions, either on our own or with others. Commercializing an autologous gene therapy such as ZYNTEGLO is resource-intensive and will require substantial investment in commercial capabilities. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without significant commercial experience as a company or the support of a third party to perform these functions, including marketing and sales functions, we may be unable to compete successfully against these more established companies. Furthermore, a significant proportion of the patient populations for ZYNTEGLO and our potential products lies outside of the United States and Europe. We may not be able to establish our global capabilities and infrastructure in a timely manner or at all. The cost of establishing such capabilities and infrastructure may not be justifiable in light of the potential revenues generated by any particular product and/or in any specific geographic region. We currently expect to rely heavily on third parties to launch and market ZYNTEGLO and our potential products in certain geographies, if approved. We may enter into collaborations with third parties to utilize their mature marketing and distribution capabilities, but we may be unable to enter into agreements on favorable terms, if at all. If our future collaborative partners do not commit sufficient resources to commercialize ZYNTEGLO or our future products, if any, and we are unable to develop the necessary commercial and manufacturing capabilities on our own, we may be unable to generate sufficient product revenue to sustain our business.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Due to the novel nature of our technology and the potential for our product to offer lifetime therapeutic benefit in a single administration, we face additional uncertainty related to pricing and reimbursement for our product. Failure to obtain or maintain adequate coverage and reimbursement for any new or current product 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 payers is essential for most patients to be able to afford expensive treatments, such as gene therapy products. In addition, because our therapies represent new treatment approaches, the estimation of potential revenues will be complex. Sales of our product and any future products will depend substantially, both domestically and abroad, on the extent to which the costs of our product and any future products 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 payers.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products, including gene therapies that are potential one-time treatments. 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, or HHS, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payers 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. 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 with the reimbursement experienced by the initial gene therapies to receive marketing authorization may create an adverse environment for reimbursement of other gene therapies.

Outside the United States, certain countries, including a number of member states of the European Union, set prices and reimbursement for pharmaceutical products, or medicinal products, as they are commonly referred to in the European Union, with limited participation from the marketing authorization holders. We cannot be sure that such prices and reimbursement will be acceptable to us or our collaborators. If the regulatory authorities in these foreign jurisdictions set prices or reimbursement levels that are not commercially attractive for us or our collaborators, the revenues from sales by us or our collaborators, and the potential profitability of our product and any future products, in those countries would be negatively affected. An increasing number of countries are taking initiatives to attempt to reduce large budget deficits by focusing cost-cutting efforts on pharmaceuticals for their state-run health care systems. These international price control efforts have impacted all regions of the world, but have been most drastic in the European Union. Additionally, some countries require approval of the sale price of a product before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then may experience delays in the reimbursement approval of our product or be subject to price regulations that would delay our commercial launch of the product, possibly for lengthy time periods, which could negatively impact the revenues we are able to generate from the sale of the product in that particular country.

Moreover, increasing efforts by governmental and third-party payers, 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 or any future products. We expect to experience pricing pressures in connection with the sale of our product and any future products, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. Net prices for drugs may be reduced by mandatory discounts or rebates required by government or private payers and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. 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.

Furthermore, because our target patient populations are relatively small, the pricing and reimbursement of our product and any future products must be adequate to cover the costs to treat and support the treatment of patients. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product and any future products will be adversely affected. 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.

In addition, the administration of our products requires procedures for the collection of HSCs from the patient, followed by chemotherapy and myeloablative treatments, before infusion of the engineered cell therapy product. The manner and level at which reimbursement is provided for these services is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our product.

We have proposed novel payment models, including outcomes-based arrangements with payments over time, to assist with realizing the value and sharing the risk of a potential one-time treatment, such as ZYNTEGLO. While we are engaged in discussions with payers, there is no assurance that there will be widespread adoption of these payment models by payers. These payment models may not be sufficient for payers to grant coverage, and if we are unable to obtain adequate coverage for our product or any future products, the adoption of our product or any future products may be limited. In addition, to the extent reimbursement for our product is subject to outcomes-based arrangements, the total payments received from product sales may vary, our cash collection of future payments and revenue assumptions from product sales will be at risk, and the timing of revenue recognition may not correspond to the timing of cash collection. We plan on commercializing our product candidates in the United States once approved, and will be subject to price reporting obligations set forth by CMS. To the extent reimbursement for our product or any future products by U.S. governmental payers is subject to outcomes-based arrangements, the increased complexity increases the risk that CMS may disagree with the assumptions and judgments that we use in our price reporting calculations, which may result in significant fines and liability.

Collectively, these factors could affect our ability to successfully commercialize our product and any future products and generate revenues, which would adversely impact our business, financial condition, results of operations and prospects.

Risks related to the research and development of our product candidates

We cannot predict when or if we will obtain marketing approval to commercialize our product candidates, and the marketing approval of our product and any future products may ultimately be for more narrow indications than we expect. If our product candidates are not approved in a timely manner or at all for any reason, our business prospects, results of operations, and financial condition would be adversely affected.

Before obtaining marketing approval from regulatory authorities for the commercialization 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. 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. 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;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical study operations or study sites or due to unforeseen safety issues;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;
- failure to obtain sufficient cells from patients to manufacture enough drug product or achieve target cell doses;
- 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.

Furthermore, the timing of our clinical studies depends on the speed at which we can recruit eligible patients to participate in testing our product candidates. The conditions for which we plan to evaluate our current product candidates in severe genetic diseases are rare disorders with limited patient pools from which to draw for clinical studies. The eligibility criteria of our clinical studies will further limit the pool of available study participants, and the process of finding and diagnosing patients may prove costly. Patients may be unwilling to participate in our 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. 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. We have experienced delays in some of our clinical studies in the past, and we may experience similar delays in the future.

Results from previous or ongoing studies are not necessarily predictive of our future clinical study results, and initial or interim results may not continue or be confirmed upon completion of the study. There is limited data concerning long-term safety and efficacy following treatment with our gene therapy and T cell-based product candidates. These data, or other positive data, may not continue or occur for these patients or for any future patients in our ongoing or future clinical studies, and may not be repeated or observed in ongoing or future studies involving our product candidates. Furthermore, 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 can be no assurance that any of these studies will ultimately be successful or support further clinical advancement or marketing approval of our product candidates. For instance, while patients with SCD who have been treated with LentiGlobin may experience a reduction of vaso-occlusive events following successful engraftment, there can be no assurance that they will not experience vaso-occlusive events in the future. Similarly, patients with relapsed and refractory multiple myeloma who have been treated with ide-cel or the bb21217 product candidate may experience disease progression. We have experienced unexpected results in the past, and we may experience unexpected results in the future. For instance, initial results from our clinical studies of ZYNTEGLO suggested that patients with TDT who do not have a β^0/β^0 genotype experienced better outcomes to treatment than patients with TDT who have a β^0/β^0 genotype. Consequently, we received conditional approval in the European Union initially for the treatment of patients with TDT who do not have a β^0/β^0 genotype. In order to support an application for marketing approval of ZYNTEGLO in patients with TDT who have a β^0/β^0 genotype, we initiated the HGB-212 study, but we do not know if or when ZYNTEGLO may be commercially available to all genotypes of TDT or types of β-thalassemia in Europe.

Even if our product candidates demonstrate safety and efficacy in clinical studies, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development. We may experience delays or rejections based upon additional government regulation from future legislation or administrative action, changes in regulatory agency policy, or additional regulatory feedback or guidance 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 marketing approval for the desired age ranges, our business may suffer. Furthermore, approvals by the EMA and the European Commission may not be indicative of what the FDA may require for approval.

In general, the FDA requires the successful completion of two pivotal trials to support approval of a biologics licensing application, or BLA, but in certain circumstances, will approve a BLA based on only one pivotal trial. Because beti-cel has been granted the FDA's Fast Track and Breakthrough Therapy designations, we are engaged in discussions with the FDA regarding the development plans for beti-cel to enable a submission of a BLA prior to the completion of our ongoing studies. Based on these discussions, we believe the results from our ongoing Northstar-2 and Northstar-3 clinical studies, together with data from our Northstar study, the LTF-303 long-term follow up protocol, and completed HGB-205 study, could be sufficient to form the basis for a BLA submission for beti-cel to treat patients with TDT. However, it should be noted that our ability to submit and obtain approval of a BLA is ultimately an FDA review decision, which will be dependent upon the data available at such time, and the available data may not be sufficiently robust from a safety and/or efficacy perspective to support the submission or approval of a BLA. Depending on the outcome of these ongoing clinical studies, the FDA may require that we conduct additional or larger pivotal trials before we can submit or obtain approval of a BLA for beti-cel for the treatment of patients with TDT. Furthermore, we are required to submit data relating to certain release assays designed to confirm the quality, purity and strength (including potency) of beti-cel as a condition for completing the BLA submission, which has the potential for further delaying the completion of our BLA submission, with the potential consequence of delaying any approval and commercial launch of beti-cel in the United States.

Based on our discussions with the FDA and EMA, we believe that we may be able to seek approval for eli-cel for the treatment of patients with CALD on the basis of safety and efficacy data from our ongoing Starbeam study, safety data from our ongoing ALD-104 study, and the completed ALD-103 observational study. Our regulatory submission plans are contingent upon eli-cel demonstrating sufficient efficacy and safety in the Starbeam study. Whether eli-cel is eligible for approval will ultimately be determined at the discretion of the FDA and EMA, and will be dependent upon the data available at such time, and the available data may not be sufficiently robust from a safety and/or efficacy perspective to support approval. Depending on the outcome of our ongoing studies, the FDA in the United States and EMA and European Commission in the European Union may require that we conduct additional or larger clinical trials before eli-cel is eligible for approval.

Based on our discussions with the FDA, we believe that we may be able to seek accelerated approval for our LentiGlobin for SCD product candidate in the United States on the basis of clinical data from Group C of our ongoing HGB-206 clinical study, with a potential first submission in the second half of 2021, and with our ongoing HGB-210 clinical study providing confirmatory data for full approval. We cannot be certain that data from our HGB-206 and HGB-210 clinical studies will be sufficiently robust from a safety and/or efficacy perspective to support full approval. We are also engaged with the EMA in discussions regarding our proposed development plans for LentiGlobin for SCD in Europe. Our development plan in the United States is contingent upon LentiGlobin for SCD demonstrating complete resolution of severe vaso-occlusive events, with globin response as a key secondary endpoint, and an acceptable safety profile in the study participants. Depending on the outcome of our ongoing and planned studies, the FDA may require that we conduct additional or larger clinical trials before our LentiGlobin product candidate is eligible for approval for the treatment of patients with SCD. The FDA may not agree with our plans for demonstrating comparability of the adherent manufacturing process to the suspension manufacturing for lentiviral vector, which may result in delays in our ability to submit a BLA for regulatory approval of LentiGlobin for SCD. In addition, we are engaged with the EMA in discussions regarding our proposed development plans for LentiGlobin in SCD in Europe, and we cannot be certain that our HGB-206 study and HGB-210 study will be sufficient to form the basis for an initial MAA submission in Europe for the treatment of patients with SCD.

In July 2020, BMS resubmitted the BLA for ide-cel as a treatment for relapsed and refractory multiple myeloma. This resubmission provided further details to address outstanding regulatory requests received from the FDA in May 2020 following the original BLA submission from March 2020. Upon preliminary review of the original submission, the FDA determined that the Chemistry, Manufacturing and Control module of the BLA required further detail to commence their review. There is no assurance that the FDA will accept the BLA resubmission. The resubmission resets the FDA review period for the BLA. Even if the FDA accepts the resubmission of the BLA for ide-cel, there is no guarantee that the FDA will conclude that the

information in such a resubmission will be sufficient to support approval and we may fail to obtain regulatory approval in the United States for ide-cel. Additionally, certain factors beyond our and BMS' control may impact the timeliness of the regulatory reviews of our submissions or any applications for approval.

If our product candidates are ultimately not approved for any reason, our business, prospects, results of operations and financial condition would be adversely affected.

**** Changes in our manufacturing processes may cause delays in our clinical development and commercialization plans.***

The manufacturing processes for our lentiviral vectors and our drug products are complex. We explore improvements to our manufacturing processes on a continual basis, as we evaluate clinical and manufacturing data and based on discussions with regulatory authorities. In some circumstances, changes in the manufacturing process may require us to perform additional comparability studies, collect additional data from patients, submit additional regulatory filings, or comply with additional requirements, which may lead to delays in our clinical development and commercialization plans. For instance, following the conditional approval of ZYNTEGLO by the European Commission, we continued to refine our commercial drug product manufacturing process to narrow some of the manufacturing process parameters and to tighten the range of commercial drug product release specifications, based on an ongoing discussion with the EMA and evolving clinical data. Implementing these changes to the ZYNTEGLO commercial manufacturing process had the effect of delaying our ability to treat the first patient in the commercial context in Europe. In LentiGlobin for SCD, we plan to seek regulatory approval for drug product utilizing lentiviral vector manufactured using the scalable suspension manufacturing process, rather than the adherent manufacturing process. The FDA and EMA may not agree with our proposed plans for demonstrating the comparability of the two processes, and may require us to conduct additional studies, collect additional data, develop additional assays, or modify release specifications, which may delay our ability to submit a BLA or MAA for regulatory approval of LentiGlobin for SCD. Over time, we also intend to transition the lentiviral vector manufacturing process for ZYNTEGLO in the European Union, and beti-cel in the United States, to the suspension manufacturing process, and the timing in which we are able to make the transition will be dependent upon reaching agreement with regulatory authorities.

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 and any future products. If our competitors obtain orphan drug exclusivity for products that regulatory authorities determine constitute the same drug and treat the same indications as our product or any future products, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

We are engaged in the development of gene therapies for severe genetic diseases and cancer, and both fields are competitive and rapidly changing. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff, manufacturing capabilities, 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, safer, or less costly than any products that we may develop, or achieve patent protection, marketing approval, product commercialization and market penetration earlier than us. Additionally, technologies developed by our competitors may render our potential products uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors. For additional information regarding our competition, see "Item 1. Business—Competition" in our Annual Report on Form 10-K.

Even if we are successful in achieving marketing 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 pathway could allow competitors to reference data from biological products already approved after 12 years from the time of approval. 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 ZYNTEGLO and 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. Generally, if a product with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the European Commission from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we will be precluded from receiving marketing approval for our product for the exclusivity period for the applicable indication.

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.

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 platform technologies, including our gene editing technology and cancer immunotherapy capabilities. Our research programs in oncology and severe genetic diseases may fail to identify other potential product candidates for clinical development for a number of reasons. We 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. 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. If any of these events occur, we may be forced to abandon our research, development or commercialization efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations.

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, and the commercial potential of our product and any future products will be materially and negatively impacted.

A significant risk in any gene therapy product based on viral vectors is that the vector will insert in or near cancer-causing oncogenes leading to uncontrolled clonal proliferation of mature cancer cells in the patient. In published studies, lentiviral vectors have demonstrated an improved safety profile over gamma-retroviral vectors used in early gene therapy studies, with no disclosed 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 we have observed in one patient from the ALD-102 study of eli-cel, now enrolled in the long-term follow-up protocol, a prevalent HSC clone with integration sites at the ACER, RFX3 and MECOM genes. An increased monitoring plan for this patient has been implemented, and as of a data cut-off date in January 2020, there have been no adverse clinical consequences of this event and the patient remains clinically stable. Similarly, in a phase 1/2 study of HPV569, which utilized an earlier generation lentiviral vector of the vector used in ZYNTEGLO and in LentiGlobin for SCD, we initially observed in one subject 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 seven years since the observation was made. The presence of the HMGA2 clone has steadily declined in this subject over time to the point that it is no longer the most common clone observed in this subject.

Notwithstanding the historical data regarding the potential safety improvements of lentiviral vectors, 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 clinical

studies or in the commercial setting. 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, and we may be unable to continue to commercialize our approved product.

Furthermore, treatment with our gene therapy product and product candidates involve chemotherapy or myeloablative treatments, which can cause side effects or adverse events that are unrelated to our product and product candidates but may still impact the perception of the potential benefits of our product and any future products. For instance, in our ongoing HGB-206 study of LentiGlobin for SCD, a serious adverse event of myelodysplasia syndrome, or MDS, was reported in a patient treated with LentiGlobin for SCD, for which there was no evidence of lentiviral-mediated oncogenesis, however MDS is a known risk of certain myeloablative regimens, and other patients receiving our product or product candidates may develop MDS in the future, which may negatively impact the commercial prospects of our product or product candidates. Additionally, our product and any future products, or procedures associated with the administration of our product or collection of patients' cells, 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, or the progression of their disease. Any of these events could impair our ability to commercialize our product and any future products and the commercial potential of our products will be materially and negatively impacted.

Patients receiving T cell-based immunotherapies, such as ide-cel and the bb21217 product candidate, may experience serious adverse events, including neurotoxicity and cytokine release syndrome. If our product candidates are revealed to have high and unacceptable severity and/or prevalence of side effects or unexpected characteristics, their clinical development, marketing approval, and commercial potential will be negatively impacted, which will significantly harm our business, financial condition and prospects.

Ide-cel and the bb21217 product candidate are chimeric antigen receptor, or CAR, T cell-based immunotherapies. In previous and ongoing clinical studies involving CAR T cell products, including those involving ide-cel and the bb21217 product candidate, patients experienced side effects such as neurotoxicity and cytokine release syndrome. There have been life threatening events related to severe neurotoxicity and cytokine release syndrome, requiring intense medical intervention such as intubation or pressor support, and in several cases, resulted in death. Severe neurotoxicity is a condition that is currently defined clinically by cerebral edema, confusion, drowsiness, speech impairment, tremors, seizures, or other central nervous system side effects, when such side effects are serious enough to lead to intensive care. In some cases, severe neurotoxicity was thought to be associated with the use of certain lymphodepletion regimens used prior to the administration of the CAR T cell products. Cytokine release syndrome is a condition that is currently defined clinically by certain symptoms related to the release of cytokines, which can include fever, chills, low blood pressure, when such side effects are serious enough to lead to intensive care with mechanical ventilation or significant vasopressor support. The exact cause or causes of cytokine release syndrome and severe neurotoxicity in connection with treatment of CAR T cell products is not fully understood at this time. In addition, patients have experienced other adverse events in these studies, such as a reduction in the number of blood cells (in the form of neutropenia, thrombocytopenia, anemia or other cytopenias), febrile neutropenia, chemical laboratory abnormalities (including elevated liver enzymes), and renal failure.

Undesirable side effects caused by ide-cel or the bb21217 product candidate, other CAR T product candidates targeting BCMA, or our other T cell-based immunotherapy product candidates, could cause us or regulatory authorities to interrupt, delay or halt clinical studies and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or other comparable foreign regulatory authorities. In some cases, side effects such as neurotoxicity or cytokine release syndrome have resulted in clinical holds of ongoing clinical trials and/or discontinuation of the development of the product candidate. Results of our studies could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the studies or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff, as toxicities resulting from T cell-based immunotherapies are not normally encountered in the general patient population and by medical personnel. Medical personnel may need additional training regarding T cell-based immunotherapy product candidates to understand their side effects. Inadequate training in recognizing or failure to effectively manage the potential side effects of T cell-based immunotherapy product candidates could result in patient deaths. Any of these occurrences may harm our business, financial condition and prospects significantly.

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

Public perception may be influenced by claims that gene therapy, including gene editing technologies, is unsafe or unethical, and research activities and adverse events in the field, even if not ultimately attributable to us or our product or product candidates, could result in increased governmental regulation, unfavorable public perception, challenges in recruiting patients to participate in our clinical studies, potential regulatory delays in the testing or approval of our potential products, stricter labeling requirements for those product candidates that are approved, and a decrease in demand for any such product. 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 approved products.

Risks related to our reliance on third parties

We are dependent on BMS for the successful development and commercialization of ide-cel and bb21217. If BMS does not devote sufficient resources to the development of ide-cel and bb21217, is unsuccessful in its efforts, or chooses to terminate its agreements with us, our business will be materially harmed.

We are co-developing and co-promoting ide-cel in the United States with BMS under our amended and restated co-development and co-promotion agreement with BMS, or the Ide-cel CCPS. Under the Ide-cel CCPS, we and BMS share the obligation to develop and commercialize ide-cel in the United States, and we will be solely dependent on BMS to develop and commercialize ide-cel outside of the United States. In addition, we have exclusively licensed to BMS the right to develop and commercialize the bb21217 product candidate, and we retain an option to co-develop and co-promote bb21217 in the United States under our license agreement with BMS. With respect to bb21217, we are responsible for completing the ongoing CRB-402 study, but BMS is responsible for further clinical development and commercialization costs, unless we choose to exercise our option to co-develop and co-promote bb21217 in the United States. If we exercise our option to co-develop and co-promote bb21217 in the United States, we and BMS will share the obligation to develop and commercialize bb21217 in the United States, and we will be solely dependent on BMS to develop and commercialize bb21217 outside of the United States.

In our partnership with BMS, BMS is obligated to use commercially reasonable efforts to develop and commercialize ide-cel and bb21217. BMS may determine however, that it is commercially reasonable to de-prioritize or discontinue the development of ide-cel and bb21217. These decisions may occur for many reasons, including internal business reasons (including due to the existence of other BMS programs that are potentially competitive with ide-cel and bb21217), results from clinical trials or because of unfavorable regulatory feedback. Further, on review of the safety and efficacy data, the FDA may impose requirements on one or both of the programs that render them commercially nonviable. In addition, under our agreements with BMS, BMS has certain decision-making rights in determining the development and commercialization plans and activities for the programs. We may disagree with BMS about the development strategy it employs, but we will have limited rights to impose our development strategy on BMS. Similarly, BMS may decide to seek marketing approval for, and limit commercialization of, ide-cel or bb21217 to narrower indications than we would pursue. More broadly, if BMS elects to discontinue the development of ide-cel or bb21217, we may be unable to advance the product candidate ourselves. We would also be prevented from developing or commercializing another CAR T cell-based product candidate that targets BCMA outside of our collaboration with BMS.

This partnership may not be scientifically or commercially successful for us due to a number of important factors, including the following:

- BMS has wide discretion in determining the efforts and resources that it will apply to its partnership with us. The timing and amount of any development milestones, and downstream commercial profits, milestones and royalties that we may receive under such partnership will depend on, among other things, BMS's efforts, allocation of resources and successful development and commercialization of ide-cel, bb21217 and other product candidates that are the subject of its collaboration with us.
- BMS may develop and commercialize, either alone or with others, products that are similar to or competitive with ide-cel, bb21217 and other product candidates that are the subject of its collaboration with us. For example, BMS is currently commercializing a number of its existing products, including lenalidomide and pomalidomide, for certain patients with relapsed and refractory multiple myeloma and is also developing JCAR-H125, another CAR-T product candidate targeting BCMA that it obtained through its acquisition of Juno Therapeutics, Inc. in March 2018.
- BMS may terminate its partnership with us without cause and for circumstances outside of our control, which could make it difficult for us to attract new strategic partners or adversely affect how we are perceived in scientific and financial communities.
- BMS may develop or commercialize our product candidates in such a way as to elicit litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential liability.

- BMS may not comply with all applicable regulatory requirements, or may fail to report safety data in accordance with all applicable regulatory requirements.
- If BMS were to breach its arrangements with us, we may need to enforce our right to terminate the agreement in legal proceedings, which could be costly and cause delay in our ability to receive rights back to the relevant product candidates. If we were to terminate an agreement with BMS due to BMS's breach or BMS terminated the agreement without cause, the development and commercialization of ide-cel or bb21217 product candidates that are the subject of its collaboration with us could be delayed, curtailed or terminated because we may not have sufficient financial resources or capabilities to continue development and commercialization of these product candidates on our own if we choose not to, or are unable to, enter into a new collaboration for these product candidates.

BMS may enter into one or more transactions with third parties, including a merger, consolidation, reorganization, sale of substantial assets, sale of substantial stock or other change in control, which could divert the attention of its management and adversely affect BMS's ability to retain and motivate key personnel who are important to the continued development of the programs under the strategic partnership with us. In addition, the third-party to any such transaction could determine to re-prioritize BMS's development programs such that BMS ceases to diligently pursue the development of our programs and/or cause the respective collaboration with us to terminate. There is no guarantee that BMS will place the same emphasis on the collaboration or on the development and commercialization of the ide-cel or bb21217 product candidates following the completion of its acquisition of Celgene in November 2019. The acquisition of Celgene by BMS may result in organizational and personnel changes, shifts in business focus or other developments that may have a material adverse effect on our collaboration.

We rely on third parties to conduct some or all aspects of our lentiviral vector production, drug product manufacturing, and testing, and these third parties may not perform satisfactorily.

We do not independently conduct all aspects of our lentiviral vector production, drug product manufacturing, and testing. We currently rely, and expect to continue to rely, on third parties with respect to these items, including manufacturing and testing in the commercial context.

Our reliance on these third parties for manufacturing, testing, research and development activities 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 products 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, and that our lentiviral vectors and drug products are manufactured in accordance with GMP as applied in the relevant jurisdictions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines, conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, or manufacture our lentiviral vectors and drug products in accordance with GMP, whether due to the impacts of COVID-19 or otherwise, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies and manufacturing process validation activities required to support future IND, MAA and BLA submissions and approval of our product candidates, or to support commercialization of our products, if approved. Many of our agreements with these third parties contain termination provisions that allow these third parties to terminate their relationships with us at any time. If we need to enter into alternative arrangements, our product development and commercialization activities could be delayed.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the products 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;
- the risk that these activities are not conducted in accordance with our study plans and protocols;
- 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.

We may be forced to manufacture lentiviral vector and drug product ourselves, for which we may not have the capabilities or resources, or enter into an agreement with a different manufacturer, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills required to manufacture our lentiviral vector or drug product candidates may be unique

or proprietary to the original manufacturer, and we may have difficulty or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. Any of these events could lead to clinical study delays or failure to obtain marketing approval, or impact our ability to successfully commercialize our product or any 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 product and product candidates. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our product and product candidates, are subject to extensive regulation. Some 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 and 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 or MAA on a timely basis and where required, must adhere to the FDA's or other regulator's good laboratory practices, or GLP, and GMP regulations enforced by the FDA or other regulator through facilities inspection programs. Some of our contract manufacturers have not produced a commercially-approved product and therefore have not obtained the requisite FDA or other marketing 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 marketing approval of our product and potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our 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 or other marketing approval of the products will not be granted.

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 or other regulators 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. The number of manufacturers with the necessary manufacturing capabilities is limited. In addition, an alternative manufacturer would need to be qualified through a BLA supplement or similar regulatory submission 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 and any future products, 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 revenues.

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 and other regulatory authorities' 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. 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 and other regulatory authorities may require us to perform additional clinical studies before approving any marketing applications.

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 marketing 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.

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 drug products, 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 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 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 have incurred net losses in each year since our inception in 1992, including net losses of $224.1 million for the six months ended June 30, 2020. As of June 30, 2020, we had an accumulated deficit of $2.51 billion. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to generate revenues. We have devoted significant financial resources to research and development, including our clinical and preclinical development activities, which we expect to continue for the foreseeable future. To date, we have financed our operations primarily through the sale of equity securities and, to a lesser extent, through collaboration agreements and grants from governmental agencies and charitable foundations. We do not expect to generate any product revenues until the second half of 2020 from ZYNTEGLO in the European Union for the treatment of adult and adolescent patients with TDT who do not have a β^0/β^0 genotype. Following marketing approval, our future revenues will depend upon the size of any markets in which our product and any future products have received approval, and our ability to achieve sufficient market acceptance, reimbursement from third-party payers and adequate market share for our product and any future products 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, including ide-cel, which we are co-developing with BMS;
- establish capabilities to support our commercialization efforts, including establishing a sales, marketing and distribution infrastructure in the United States and Europe, and to commercialize ZYNTEGLO and any other products for which we may obtain marketing approval;
- obtain, build and expand manufacturing capacity, including capacity at third-party manufacturers and our own manufacturing facility;
- initiate additional research, preclinical, clinical or other programs as we seek to identify and validate additional product candidates;
- acquire or in-license other product candidates and technologies;
- maintain, protect and expand our intellectual property portfolio;
- attract and retain skilled personnel; 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 revenues and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory, pricing and reimbursement approvals necessary to commercialize our product and any future products. Our ability to generate 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, commercial-scale, reproducible, and transferable manufacturing process for our vectors and drug products;
- 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 for our product candidates and commercial demand for any approved product;
- launching and commercializing any approved product, either by collaborating with a partner or, if launched independently, by establishing a field-based team, marketing and distribution infrastructure;
- obtaining sufficient pricing and reimbursement for any approved product from private and governmental payers;
- obtaining market acceptance and adoption of any approved product and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter; and
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how.

We expect to continue to incur significant expenditures for the foreseeable future, and we expect these expenditures to increase as we commercialize ZYNTEGLO in the European Union, which costs may increase with any increased competition. Our expenses could increase beyond expectations if we are required by the FDA, 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 product revenues, 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 programs in β-thalassemia, SCD, CALD, and multiple myeloma through clinical development and other product candidates through preclinical development. Developing and commercializing gene therapy products is expensive, and we expect our research and development expenses and our commercialization expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates and progress our commercialization efforts.

As of June 30, 2020, our cash, cash equivalents and marketable securities were $1.60 billion. In response to the ongoing COVID-19 pandemic and the associated economic conditions, we have revised our operating plan to execute on our strategy during this period of uncertainty. Based on our current business plan, we expect our cash, cash equivalents, and marketable securities, together with projected revenue generated under our collaborative arrangements and projected sales of products, to fund our operations into 2023. However, our revised operating plan may change further as a result of the COVID-19 pandemic and the surrounding economic conditions, as well as many other factors currently unknown to us. In addition, we may seek additional funds through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements or other collaborations, strategic alliances and licensing arrangements or a combination of these approaches, during this period. In any event, we will require additional capital to obtain marketing approval for, and to commercialize, our product candidates. 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 objectives.

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 approved product and product candidates. In addition, we cannot guarantee that 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, or if revenues from collaboration arrangements or product sales are less than we have projected, 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.

If the estimates we make, or the assumptions on which we rely, in preparing our consolidated financial statements are incorrect, our actual results may vary from those reflected in our projections and accruals.

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America, or GAAP. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. We cannot assure you, however, that our estimates, or the assumptions underlying them, will be correct. We may be incorrect in our assumptions regarding the applicability of drug pricing programs and rebates that may be applicable to our product or any future products, which may result in our under- or over-estimating our anticipated product revenues especially as applicable laws and regulations governing pricing evolve over time. In addition, to the extent payment for our product or any future products is subject to outcomes-based arrangements over time, the total payments received from product sales may vary, our cash collection of future payments and revenue assumptions from product sales will be at risk, and the timing of revenue recognition may not correspond to the timing of cash collection.

Further, from time to time we issue financial guidance relating to our expectations for our cash, cash equivalents, and marketable securities available for operations, which guidance is based on estimates and the judgment of management. If, for

any reason, our expenses differ materially from our guidance or we utilize our cash more quickly than anticipated, we may have to adjust our publicly announced financial guidance. If we fail to meet, or if we are required to change or update any element of, our publicly disclosed financial guidance or other expectations about our business, our stock price could decline.

**** Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.***

Our operating results are difficult to predict and will likely fluctuate from quarter to quarter and year to year. Due to the recent approval by the European Commission of ZYNTEGLO and the absence of historical sales data, our product sales will be difficult to predict from period to period. This uncertainty is heightened by the unpredictable scope of the impact of the COVID-19 pandemic, which has adversely affected the operations of third parties upon which we rely in our commercialization efforts, patient access to hospitals, physicians' offices, clinics and other administration sites, and global economic conditions, as well as caused a re-prioritization of healthcare services.

In addition, we have entered into licensing and collaboration agreements with other companies that include research and development funding and milestone payments to us, and we expect that amounts earned from our collaboration agreements will continue to be an important source of our revenues. Accordingly, our revenues will also depend on research and development funding and the achievement of development and clinical milestones under our existing collaboration and license agreements, including, in particular, our collaborations with BMS and Regeneron, as well as entering into potential new collaboration and license agreements. These payments may vary significantly from quarter to quarter and any such variance could cause a significant fluctuation in our operating results from one quarter to the next.

Further, changes in our operations, such as increased development, manufacturing and clinical trial expenses in connection with our expanding pipeline programs, or our undertaking of additional programs, or business activities, or entry into strategic transactions, including potential future acquisitions of products, technologies or businesses may also cause significant fluctuations in our expenses.

The cumulative effects of these factors, further exacerbated by the impacts of the ongoing COVID-19 pandemic on healthcare systems and economic conditions, will likely result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

Risks related to our business operations

****We are commercializing ZYNTEGLO outside of the United States, and therefore we will be subject to the risks of doing business outside of the United States.***

Because we are commercializing ZYNTEGLO outside of the United States, our business is subject to risks associated with doing business outside of the United States. Accordingly, our business and financial results in the future could be adversely affected due to a variety of factors, including:

- efforts to develop an international commercial and supply chain organization may increase our expenses, divert our management's attention from the acquisition or development of product candidates or cause us to forgo profitable licensing opportunities in these geographies;
- requirements or limitations imposed by a specific country or region on potential qualified treatment centers or other aspects of commercialization applicable to autologous gene therapies such as ours;
- changes in a specific country's or region's political and cultural climate or economic condition, including as a result of the COVID-19 pandemic;
- unexpected changes in foreign laws and regulatory requirements;
- difficulty of effective enforcement of contractual provisions in local jurisdictions;
- inadequate intellectual property protection in foreign countries;

- trade-protection measures, import or export licensing requirements such as Export Administration Regulations promulgated by the U.S. Department of Commerce and fines, penalties or suspension or revocation of export privileges;
- the effects of applicable foreign tax structures and potentially adverse tax consequences; and
- significant adverse changes in foreign currency exchange rates, including as a result of the United Kingdom's exit from the European Union on January 31, 2020, or Brexit.

In addition to FDA and related regulatory requirements in the United States and abroad, we are subject to extensive additional federal, state and foreign anti-bribery regulation, which include the U.S. Foreign Corrupt Practices Act, the U.K. Bribery Act, and similar laws in other countries outside of the United States. We have developed and implemented a corporate compliance program based on what we believe are current best practices in the pharmaceutical industry for companies similar to ours, but we cannot guarantee that we, our employees, our consultants or our third-party contractors are or will be in compliance with all federal, state and foreign regulations regarding bribery and corruption. Moreover, our partners and third-party contractors located outside the United States may have inadequate compliance programs or may fail to respect the laws and guidance of the territories in which they operate. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could also have an adverse effect on our business, financial condition and results of operations.

As we evolve from a U.S.-based company primarily involved in discovery, preclinical research and clinical development into a company that develops and commercializes multiple drugs with an international presence, we will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

We received conditional marketing authorization for our first product in 2019 and are launching ZYNTEGLO to treat the first patient in the commercial setting in the second half of 2020, which we hope will be the first of a sequence of marketing approvals and commercial launches for multiple products across multiple geographies. As we advance multiple product candidates through late-stage clinical research and plan submissions for marketing authorizations, we are expanding our operations in the United States and Europe. As of June 30, 2020, we had 1,167 full-time employees. As we pursue our development and commercialization strategy, we expect to expand our full-time employee base and to hire more consultants and contractors in the United States and Europe. This expected growth may place a strain on our administrative and operational infrastructure. As a result, 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. 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. Recruiting and retaining qualified employees, consultants and advisors for our business, including scientific and technical personnel, will be critical to our success. 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. 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.

Even if we receive marketing approval for a product candidate, any approved product will remain subject to regulatory scrutiny.

Even if we obtain marketing approval in a jurisdiction, regulatory authorities may still impose significant restrictions on the indicated uses or marketing of any approved products such as ZYNTEGLO, or impose ongoing requirements for potentially costly post-approval studies, post-market surveillance or patient or drug restrictions. 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 marketing approval for a product, 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 marketing 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.

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 any approved product and generate revenues.

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

In the United States, the research, manufacturing, distribution, sale, and promotion of drugs and biologic products are subject to regulation by various federal, state, and local authorities in addition to FDA, including CMS, other divisions of the HHS, (e.g., the Office of Inspector General), the United States Department of Justice offices of the United States Attorney, the Federal Trade Commission and state and local governments. Our operations are directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations described in more detail under *"Item 1. Business--Government regulation"* in our Annual Report. These include the federal Anti-Kickback Statute, federal civil and criminal false claims laws and civil monetary penalty laws (including False Claims Laws), HIPAA, transparency requirements created under the Affordable Care Act, as well as analogous state and foreign laws.

These laws apply to, among other things, our sales, marketing and educational programs. State and federal regulatory and enforcement agencies continue actively to investigate violations of health care laws and regulations, and the United States Congress continues to strengthen the arsenal of enforcement tools. Most recently, the Bipartisan Budget Act of 2018 increased the criminal and civil penalties that can be imposed for violating certain federal health care laws, including the Anti-Kickback Statute. Enforcement agencies also continue to pursue novel theories of liability under these laws. In particular, government agencies have recently increased regulatory scrutiny and enforcement activity with respect to programs supported or sponsored by pharmaceutical companies, including reimbursement and co-pay support, funding of independent charitable foundations and other programs that offer benefits for patients. Several investigations into these programs have resulted in significant civil and criminal settlements.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our financial condition and divert the attention of our management from operating our business.

In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. For example, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, imposes requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and

transmission of individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions In addition to HIPAA, as amended by HITECH, and their respective implementing regulations, California recently enacted the California Consumer Privacy Act, or CCPA, which creates new individual privacy rights for California consumers (as defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA will require covered companies to provide certain disclosures to consumers about its data collection, use and sharing practices, and to provide affected California residents with ways to opt-out of certain sales or transfers of personal information. The CCPA went into effect on January 1, 2020, and the California Attorney General will commence enforcement actions against violators beginning July 1, 2020. While there is currently an exception for protected health information that is subject to HIPAA, as currently written, the CCPA may impact our business activities. The California Attorney General has proposed draft regulations, which have not been finalized to date, that may further impact our business activities if they are adopted. The uncertainty surrounding the implementation of CCPA exemplifies the vulnerability of our business to the evolving regulatory environment related to personal data and protected health information.

In the European Union, interactions between pharmaceutical companies, healthcare professionals, and patients are also governed by strict laws, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct in the individual EU member states. The provision of benefits or advantages to healthcare professionals to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. Also, direct-to-consumer advertising of prescription-only medicinal products is prohibited at the European Union level and in the individual member states. In addition, the UK Bribery Act applies to any company incorporated in or "carrying on business" in the UK, irrespective of where in the world the alleged bribery activity occurs, which could have implications for our interactions with physicians both in and outside of the UK. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union member states must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union member states. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union member states. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

EU member states, Switzerland and other countries have also adopted data protection laws and regulations, which impose significant compliance obligations. In the European Union, the collection and use of personal health data is currently governed by the provisions of the General Data Protection Regulation, or the GDPR. The GDPR, together with the national legislation of the individual EU member states governing the processing of personal data, impose strict obligations and restrictions on the ability to collect, analyze and transfer personal data, including health data from clinical trials and adverse event reporting. In particular, these obligations and restrictions concern the consent of the individuals to whom the personal data relates, the information provided to the individuals for the consent to be considered valid, the transfer of personal data out of the European Economic Area, security breach notifications, the use of third-party processors in connection with the processing of the personal data, confidentiality of the personal data, as well as substantial potential fines for breaches of the data protection obligations. Data protection authorities from the different EU member states may interpret the GDPR and national laws differently and impose additional requirements, which add to the complexity of processing personal data in the European Union. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR is a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with any activities falling within the scope of the GDPR. Further, Brexit has created uncertainty with regard to data protection regulation in the United Kingdom. In particular, it is unclear how data transfers to and from the United Kingdom will be regulated.

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 approved product or product candidates harms patients, or is perceived to harm patients even when such harm is unrelated to our approved product or product candidates, our marketing 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 patients participating in clinical trials, consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our product or product candidates. There is a risk that our product or 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 develop our product candidates or commercialize any approved product; and
- decreased demand for any approved product.

We carry product liability insurance and we believe our product liability insurance coverage is sufficient in light of our current clinical programs and approved product; however, we may not be able to maintain insurance coverage at commercially reasonable cost or in sufficient amounts to protect us against losses due to liability. 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 approved product and 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 approved product or 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 marketing approval for any approved product, 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 marketing approval process in other countries, or impact and limit the type of marketing approval our product candidates may receive or any approved product maintains. 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.

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

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our product candidates or any future product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product for which we obtain marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or the Affordable Care Act, was passed, which substantially changed the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Affordable Care Act, among other things, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extended the rebate program to individuals enrolled in Medicaid managed care organizations, established annual fees and taxes on manufacturers of certain

branded prescription drugs, expanded the types of entities eligible for the 340B drug discount program, and a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% (increased pursuant to the Bipartisan Budget Act of 2018, effective as of 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Since its enactment, there have been numerous judicial, administrative, executive, and legislative challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to the Affordable Care Act in the future. Various portions of the Affordable Care Act are currently undergoing legal and constitutional challenges in the Fifth Circuit Court and the United States Supreme Court. Additionally, the Trump Administration has issued various Executive Orders which eliminated cost sharing subsidies and various provisions that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices, and Congress has introduced several pieces of legislation aimed at significantly revising or repealing the Affordable Care Act. It is unclear whether the Affordable Care Act will be overturned, repealed, replaced, or further amended. We cannot predict what effect further changes to the Affordable Care Act would have on our business

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. In August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction, which triggered the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of, on average, 2% per fiscal year through 2025 unless Congress takes additional action. These reductions were extended through 2029 through subsequent legislative amendments. In January 2013, the American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration's budget proposal for fiscal years 2019 and 2020 contain further drug price control measures that could be enacted during the budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. HHS has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy, a type of prior authorization, for Part B drugs beginning January 1, 2020. This final rule codified CMS's policy change that was effective January 1, 2019. Although a number of these, and other proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. For example, on September 25, 2019, the Senate Finance Committee introduced the Prescription Drug Pricing Reduction Act of 2019, a bill intended to reduce Medicare and Medicaid prescription drug prices. The proposed legislation would restructure the Part D benefit, modify payment methodologies for certain drugs, and impose an inflation cap on drug price increases. An even more restrictive bill, the Lower Drug Costs Now Act of 2019, was passed in the House of Representatives on December 12, 2019 and sent to the Senate, and would require the Department of Health and Human Services (HHS) to directly negotiate drug prices with manufacturers. It is unclear whether either of these bills will make it through both chambers and be signed into law, and if either is enacted, what effect it would have on our business. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that the healthcare reform measures that have been adopted and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product and

could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private third-party payers.

The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize ZYNTEGLO and any other products for which we obtain marketing approval.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product. Such reforms could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop product candidates.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our clinical development programs and the diseases our product candidates are being developed to treat. We intend to utilize appropriate social media in connection with our commercialization efforts following approval of our product candidates. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to report an alleged adverse event. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations, or we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our investigational products. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website, or a risk that a post on a social networking website by any of our employees may be construed as inappropriate promotion. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business.

Our computer systems, or those of our third-party collaborators, service providers, contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product candidates' development programs and have a material adverse effect on our reputation, business, financial condition or results of operations.

Our computer systems and those of our current or future third-party collaborators, service providers, contractors and consultants may fail and are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. The size and complexity of our information technology systems, and those of our collaborators, service providers, contractors and consultants, and the large amounts of information stored on those systems make those systems vulnerable to service interruptions, security breaches, or other failures, resulting from inadvertent or intentional actions by our employees or those of third-party business partners, or from cyber-attacks by malicious third parties. Attacks on information technology systems are increasing in their frequency, levels of persistence, sophistication and intensity, and they are being conducted by increasingly sophisticated and organized groups and individuals with a wide range of motives and expertise. In addition to extracting sensitive information, such attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. The prevalent use of mobile devices also increases the risk of data security incidents. If we experience a material system failure, accident or security breach that causes interruptions in our operations or the operations of third-party collaborators, service providers, contractors and consultants, it could result in significant reputational, financial, legal, regulatory, business or operational harm. For example, the loss of clinical trial data for our product candidates could result in delays in our marketing approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or product candidates, or inappropriate disclosure of confidential or proprietary information, we could incur liabilities and the further development of our product candidates could be delayed. We also rely on third-party service providers for aspects of our internal control over financial reporting and such service providers may experience a material system failure or fail to carry out their obligations in other respects, which may impact our ability to produce accurate and timely financial statements, thus harming our operating results, our ability to operate our business, and

our investors' view of us. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to material failures, security breaches, cyberattacks and other related breaches.

Any failure or perceived failure by us or any third-party collaborators, service providers, contractors or consultants to comply with our privacy, confidentiality, data security or similar obligations to third parties, or any data security incidents or other security breaches that result in the unauthorized access, release or transfer of sensitive information, including personally identifiable information, may result in governmental investigations, enforcement actions, regulatory fines, litigation or public statements against us. These events could cause third parties to lose trust in us or could result in claims by third parties asserting that we have breached our privacy, confidentiality, data security or similar obligations, any of which could have a material adverse effect on our reputation, business, financial condition or results of operations. Moreover, data security incidents and other security breaches can be difficult to detect, and any delay in identifying them may lead to increased harm. While we have implemented data security measures intended to protect our information technology systems and infrastructure, there can be no assurance that such measures will successfully prevent service interruptions or data security incidents.

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, and 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.

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, *ex part*e reexaminations, post-grant review, and *inter partes* review proceedings before the U.S. Patent and Trademark Office, or U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. 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 product candidates and commercialize our approved product. 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.

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 the development of our product candidates or allow commercialization of our approved product, 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, approved product, 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 approved product or 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. 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 patent eligible subject matter, 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 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 approved product and/or product candidates. Such a loss of patent protection would have a material adverse impact on our business.

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.

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 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.

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

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

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 Select 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 biotechnology and pharmaceutical industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

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 our product, product candidates or other gene therapy products, or in clinical studies of such products;
- inability to obtain additional funding;
- any delay in filing an IND, MAA or BLA for any of our product candidates, including ide-cel, and any adverse development or perceived adverse development with respect to the FDA's review of that IND, MAA or BLA;
- failure to successfully manage the commercial launch of ZYNTEGLO, or our product candidates following marketing approval, including failure to manage our supply chain operations in the coordination and delivery of drug product to patients at qualified treatment centers;
- failure to obtain sufficient pricing and reimbursement for ZYNTEGLO or our product candidates from private and governmental payers;
- failure to obtain market acceptance and adoption of ZYNTEGLO or any other potential product following marketing approval;
- 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 ZYNTEGLO or 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.

****The market price of our common stock may be adversely affected by market conditions affecting the stock markets in general, including price and trading fluctuations on the Nasdaq Global Select Market.***

Market conditions may result in volatility in the level of, and fluctuations in, market prices of stocks generally and, in turn, our common stock and sales of substantial amounts of our common stock in the market, in each case being unrelated or disproportionate to changes in our operating performance. Any actual or perceived weakness in the economy in general could increase the volatility of the stock market, which may adversely affect the market price of our common stock.

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 and members of our board of directors, 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.

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 automatically increases 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. We also have an Employee Stock Purchase Plan and any shares of common stock purchased pursuant to that plan will also cause dilution.

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 biotechnology and 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.

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 have completed several financings since our inception which we believe have resulted in a change in control as defined by IRC Section 382. 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.

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.

****Changes in tax law could adversely affect our business and financial condition.***

The rules dealing with U.S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. For example, on March 27, 2020, President Trump signed into law the "Coronavirus Aid, Relief, and Economic Security Act" or the CARES Act, which included certain changes in tax law intended to stimulate the U.S. economy in light of the COVID-19 coronavirus outbreak, including temporary beneficial changes to the treatment of net operating losses, interest deductibility limitations and payroll tax matters. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

Item 2. Unregistered Sales of Equity Securities and Uses of Proceeds

None

Item 3. Defaults Upon Senior Securities

None

Item 4. Mine Safety Disclosures

None

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 Jason Cole (Chief Operating and Legal Officer), David Davidson (Chief Medical Officer), and Kory Wentworth (Vice President, Finance and Treasurer)) 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 in the Exhibit Index below, which is incorporated herein by reference.

Exhibit Index

Exhibit Number	Exhibit Title	Incorporated by Reference			
		Form	File no.	Exhibit	Filing Date
2.1	Stock Purchase Agreement by and between the Registrant and Precision Genome Engineering, Inc.	8-K	001-35966	2.1	June 30, 2014
3.1	Amended and Restated Certificate of Incorporation of the Registrant	8-K	001-35966	3.1	June 24, 2013
3.2	Amended and Restated By-laws of the Registrant	8-K	001-35966	3.2	June 24, 2013
3.3	Amendment No. 1 to Amended and Restated By-laws of the Registrant	8-K	001-35966	3.1	February 11, 2016
4.1	Specimen Common Stock Certificate	S-1/A	333-188605	4.1	June 4, 2013
10.1#	Second Amended and Restated 2002 Employee, Director and Consultant Stock Plan, as amended, and forms of award agreement thereunder	S-1	333-188605	10.1	May 14, 2013
10.2#	2010 Stock Option and Grant Plan, as amended, and forms of award agreement thereunder	S-1	333-188605	10.2	May 14, 2013
10.3#	2013 Stock Option and Incentive Plan and forms of award agreement thereunder	S-1/A	333-188605	10.3	June 4, 2013
10.4	Form of Indemnification Agreement between the Registrant and each of its Executive Officers and Directors	S-1	333-188605	10.4	May 14, 2013
10.5†	Patent License Agreement, dated December 11, 1996, by and between the Registrant (formerly known as Genetix Pharmaceuticals Inc., successor-in-interest to Innogene Pharmaceuticals, Inc.) and Massachusetts Institute of Technology, as amended	S-1	333-188605	10.6	May 14, 2013
10.6†	Fourth Amendment to Patent License Agreement, dated October 28, 2016, by and between the Registrant and Massachusetts Institute of Technology	10-K	001-35966	10.7	February 22, 2017
10.7†	Patent and Know-How License Agreement No. 07554F30, dated May 14, 2009, by and between the Registrant (formerly known as Genetix Pharmaceuticals Inc.) and INSERM-TRANSFERT, as amended	S-1	333-188605	10.7	May 14, 2013
10.8†	License Agreement, dated September 13, 2011, by and between the Registrant and Institut Pasteur, as amended	S-1	333-188605	10.8	May 14, 2013
10.9†	Amendment No. 3 to License Agreement, dated September 10, 2013, by and between the Registrant and Institut Pasteur	10-Q	001-35966	10.2	November 14, 2013
10.10†	Amendment No. 4 to License Agreement, dated April 1, 2015, by and between the Registrant and Institut Pasteur	10-Q	001-35966	10.1	May 6, 2015
10.11†	License Agreement, dated December 7, 2011, by and between the Registrant and Research Development Foundation	S-1	333-188605	10.9	May 14, 2013
10.12†	Novation Agreement, dated April 2, 2012, by and between the Registrant and The Board of Trustees of the Leland Stanford Junior University	S-1	333-188605	10.1	May 14, 2013
10.13†	Master Collaboration Agreement by and between the Registrant and Celgene Corporation, dated March 19, 2013	S-1	333-188605	10.11	May 14, 2013
10.14†	Amended and Restated Master Collaboration Agreement by and between the Registrant and Celgene Corporation, dated June 3, 2015	10-Q	001-35966	10.14	August 7, 2015
10.15	Amendment No. 1 to Amended and Restated Master Collaboration Agreement by and between the Registrant and Celgene Corporation, dated February 17, 2016	10-Q	001-35966	10.15	May 4, 2016
10.16	Amendment No. 2 to Amended and Restated Master Collaboration Agreement by and between the Registrant and Celgene Corporation, dated September 28, 2017	10-Q	001-35966	10.17	November 1, 2017
10.17†	Amended and Restated License Agreement by and between the Registrant and Celgene Corporation, dated February 16, 2016	10-Q/A	001-35966	10.16	November 2, 2016

10.18††	Second Amended and Restated License Agreement by and between the Registrant and Celgene Corporation and Celgene European Investment Company LLC, dated May 8, 2020	--	--	--	Filed herewith
10.19†	Amended and Restated Co-Development, Co-Promote and Profit Share Agreement by and between the Registrant and Celgene Corporation and Celgene European Investment Company LLC, dated March 26, 2018	10-Q	001-35966	10.2	May 2, 2018
10.20††	First Amendment to Amended and Restated Co-Development, Co-Promote and Profit Share Agreement by and between the Registrant and Celgene Corporation and Celgene European Investment Company LLC, dated May 8, 2020	--	--	--	Filed herewith
10.21†	License Agreement by and between the Registrant and Biogen Idec MA Inc., dated August 13, 2014	10-Q/A	001-35966	10.17	November 2, 2016
10.22†	Letter Agreement by and between the Registrant and Biogen MA Inc., dated September 29, 2017	10-Q	001-35966	10.21	November 1, 2017
10.23†	Exclusive Patent License Agreement by and between the Registrant and the National Institutes of Health, dated August 31, 2015	10-Q/A	001-35966	10.18	November 2, 2016
10.24†	License Agreement, dated December 23, 2015, by and between the Registrant and SIRION Biotech GmbH	10-K	001-35966	10.23	February 21, 2019
10.25††	Toll Manufacturing and Service Agreement, dated November 18, 2016 by and between the Registrant and APCETH Biopharma GmbH, as amended	10-Q	001-35966	10.24	August 1, 2019
10.26††	Amendment Agreement No. 3 to the Toll Manufacturing and Service Agreement by and between bluebird bio (Switzerland) GmbH and Apceth Biopharma GmbH	8-K	001-35966	10.1	March 12, 2020
10.27††	Clinical and Commercial Supply Agreement – Viral Vector Product, dated November 27, 2017, by and between the Registrant and SAFC Carlsbad, Inc., as amended	10-Q	001-35966	10.25	August 1, 2019
10.28††	Amendment No. 2 to Clinical and Commercial Supply Agreement Viral Vector Product by and between bluebird bio (Switzerland) GmbH and SAFC Carlsbad, Inc.	8-K	001-35966	10.1	January 21, 2020
10.29#	Amended and Restated Employment Agreement by and between the Registrant and Nick Leschly	S-1/A	333-188605	10.12	June 4, 2013
10.30#	Amended and Restated Employment Agreement by and between the Registrant and Jeffrey T. Walsh	S-1/A	333-188605	10.13	June 4, 2013
10.31#	Amended and Restated Employment Agreement by and between the Registrant and David M. Davidson, M.D.	S-1/A	333-188605	10.15	June 4, 2013
10.32#	Employment Agreement, dated February 3, 2014, by and between the Registrant and Jason F. Cole	10-Q	001-35966	10.18	May 13, 2014
10.33#	Amendment to Employment Agreement, dated March 7, 2016, by and between the Registrant and Jason F. Cole	10-Q	001-35966	10.25	May 4, 2016
10.34#	Amendment No. 2 to Employment Agreement, dated November 3, 2016, by and between the Registrant and Jason F. Cole	10-K	001-35966	10.27	February 22, 2017
10.35#	Employment Agreement, dated May 30, 2015, by and between the Registrant and Philip D. Gregory	10-Q	001-35966	10.21	August 7, 2015
10.36#	Amendment to Employment Agreement, dated November 3, 2016, by and between the Registrant and Philip D. Gregory	10-K	001-35966	10.31	February 22, 2017
10.37#	2013 Employee Stock Purchase Plan	S-1/A	333-188605	10.17	June 4, 2013
10.38#	First Amendment of the Bluebird Bio, Inc. 2013 Employee Stock Purchase Plan	10-K	001-35966	10.38	February 21, 2018
10.39#	Offer Letter, dated November 16, 2017, by and between the Registrant and Kory Wentworth	10-K	001-35966	10.39	February 21, 2018
10.40#	Executive Cash Incentive Bonus Plan	S-1	333-188605	10.18	May 14, 2013
10.41#	Employment Agreement, dated December 18, 2018, by and between the Registrant and William (“Chip”) Baird	8-K	001-35966	10.1	February 11, 2019

10.42†	Lease, dated September 21, 2015, by and between the Registrant and ARE-MA Region No. 40 LLC	10-Q	001-35966	10.3	November 5, 2015
10.43	First Amendment to Lease, dated June 21, 2016, by and between the Registrant and ARE-MA Region No. 40 LLC	10-Q	001-35966	10.37	August 3, 2016
10.44	Second Amendment to Lease, dated November 14, 2016, by and between the Registrant and ARE-MA Region No. 40 LLC	10-K	001-35966	10.44	February 22, 2017
10.45††	Sublease, dated April 16, 2019, by and between the Registrant and Aventis Inc.	10-Q	001-35966	10.42	August 1, 2019
10.46	Amendment to Sublease, dated April 19, 2019, by and between the Registrant and Aventis Inc.	10-Q	001-35966	10.43	August 1, 2019
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.	—	—	—	Filed herewith
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.	—	—	—	Filed herewith
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.	—	—	—	Furnished herewith
101.INS	Inline XBRL Instance Document (the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document)				
101.SCH	Inline XBRL Taxonomy Extension Schema Document.	—	—	—	Filed herewith
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.	—	—	—	Filed herewith
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.	—	—	—	Filed herewith
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.	—	—	—	Filed herewith
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.	—	—	—	Filed herewith
104	Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibits 101)	—	—	—	Filed herewith

† 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.

†† Portions of this exhibit (indicated by asterisks) have been omitted in accordance with the rules of the SEC.

# Indicates a management contract or any compensatory plan, contract or arrangement.

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: August 5, 2020

By: /s/ Nick Leschly

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

Date: August 5, 2020

By: /s/ Chip Baird

Chip Baird
Chief Financial Officer (Principal Financial Officer and Duly Authorized Officer)

Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

Exhibit 10.18

Execution Version

Second Amended and Restated License Agreement

by and between

bluebird bio, Inc.

and

Celgene Corporation

and

Celgene European Investment Company LLC

May 8, 2020

Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

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Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

List of Appendices

Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

Second Amended and Restated License Agreement

This Second Amended and Restated License Agreement (this "License Agreement"), dated as of May 8, 2020 (the "Amendment Effective Date"), is made by and between bluebird bio, Inc., a Delaware corporation ("Bluebird"), and Celgene Corporation, a Delaware Corporation ("Celgene Corp"), with respect to all rights and obligations under this License Agreement in the United States (subject to Section 11.18), and Celgene European Investment Company LLC, a Delaware limited liability company, with respect to all rights and obligations under this License Agreement outside of the United States (subject to Section 11.18) ("Celgene Europe" and together with Celgene Corp, "Celgene"). Each of Bluebird and Celgene may be referred to herein as a "Party" or together as the "Parties."

WHEREAS, Bluebird has developed and owns or has rights to certain Patents and technology relating to developing innovative gene therapies for genetic disorders;

WHEREAS, Celgene is a biopharmaceutical company focused on acquiring, Developing and Commercializing innovative anti-cancer agents;

WHEREAS, Bluebird and Celgene were parties to that certain Master Collaboration Agreement, dated as of March 19, 2013, pursuant to which the Parties entered into a global strategic collaboration to research, develop and commercialize therapeutic products in the Field (the "Original MCA");

WHEREAS, the Parties entered into an Amended and Restated Collaboration Agreement, dated as of June 3, 2015 (as amended from time to time, the "Master Collaboration Agreement"), pursuant to which the Parties amended and restated the Original MCA in order to continue the research and development of the Product Candidates pursuant to the terms set forth therein;

WHEREAS, pursuant to the terms of the Master Collaboration Agreement, Celgene has exercised its option to select a Product Candidate to be an Optioned Candidate by delivering to Bluebird a Celgene Option Notice and payment of the applicable Initial Option Fee and Additional Option Fee (such Optioned Candidate, as defined more fully in Appendix A, the "Elected Candidate");

WHEREAS, effective as of September 28, 2017 (the "Original License Agreement Effective Date"), the Parties entered into an Amended and Restated License Agreement whereby Celgene obtained exclusive rights to Develop Elected Candidate and Commercialize Licensed Product (the "Original Agreement");

WHEREAS, the Parties entered on the date hereof into a First Amendment to the Amended and Restated Co-Development, Co-Promote and Profit Share Agreement dated as March 26, 2018 that provided for a payment to Bluebird the partial consideration of which was the entry into of this License Agreement; and

WHEREAS, the Parties wish to amend and restate certain terms of the Original Agreement, including with respect to the Manufacture and supply of Vectors, payments and royalties and exclusivity in accordance with the terms and conditions set forth below on the terms and conditions set forth below.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and for other good and valuable consideration, the amount and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

1. Definitions.

The following terms and their correlatives will have the meanings set forth below. Capitalized terms used, but not defined, herein will have the meanings ascribed to such terms in the Master Collaboration Agreement.

1.1 "Applicable Bluebird In-Licenses" means the Applicable Pre-Existing In-Licenses and the Applicable New In-Licenses.

1.2 "Applicable New In-Licenses" means all New In-Licenses of Bluebird or its Affiliates necessary or useful for the research, Development and/or Commercialization of Elected Candidate and Licensed Product that Celgene has elected to list on Appendix B as of the Original License Agreement Effective Date, plus any other New In-License of Bluebird or its Affiliates that Celgene has elected to include as an Applicable New In-License pursuant to Section 3.2(b).

1.3 "Applicable Pre-Existing In-Licenses" means all Pre-Existing In-Licenses necessary or useful for the research, Development and/or Commercialization of Elected Candidate and Licensed Product, and any extensions or expansions of the scope of such Pre-Existing In-Licenses, including those listed on Appendix C.

1.4 "Biosimilar Product" means, with respect to a Licensed Product in any country, any biosimilar product sold by a Third Party not authorized by or on behalf of Celgene, its Affiliates or Sublicensees, (a) that is a biosimilar biological product, as defined in 21 USC 379j-51 (or any successor or replacement thereof), a similar biological medicinal product, as defined in Annex I to Directive 2001/83/EC (or any successor or replacement thereof), or any similar biosimilar or generic product under the Laws of any country or jurisdiction, or (b) regarding which Regulatory Approval is obtained by referencing Regulatory Data of such Licensed Product.

1.5 "Bluebird In-Licensed IP" means all Patents, Materials and Know-How in-licensed by Bluebird pursuant to Applicable Bluebird In-Licenses, including any extensions or expansions of the scope thereof.

1.6 "Bluebird Technology" means all Bluebird Solely Owned IP and all of Bluebird's right, title and interest in and to Joint IP.

1.7 "Celgene Development & Commercialization Program" means a Development and Commercialization program for Licensed Product in the Field worldwide.

1.8 "Celgene Licensed Product In-License" means any Applicable Celgene In-License or other agreement between Celgene or any of its Affiliates and a Third Party entered into under Section 4.3(d) pursuant to which Celgene or any of its Affiliates in-licenses any Know-How, Materials or Patents that directly relate to or Cover the Elected Candidate and/or Licensed Product or its Manufacture or use.

1.9 "Celgene Licensed Product In-Licensed IP" means any Patents, Materials and Know-How Controlled at any time during the License Agreement Term by Celgene or any of its Affiliates pursuant to a Celgene Licensed Product In-License or Celgene Other In-License that directly relate to or Cover the Elected Candidate and/or Licensed Product or its Manufacture or use.

1.10 "Celgene Licensed Product IP" means (a) Celgene Technology, (b) Collaboration IP solely owned by Celgene and Celgene's interest in jointly owned Collaboration IP, and (c) Patents, Materials or Know-How (to the extent not included in subsection (a) or (b)) owned by Celgene or its Affiliates that are Controlled at any time during the License Agreement Term by Celgene or any of its Affiliates, in each case that directly relate to or Cover the Elected Candidate and/or Licensed Product or its Manufacture or use.

1.11 "Celgene Other In-License" means any agreement between Celgene or any of its Affiliates and a Third Party, other than Applicable Celgene In-Licenses and any agreement between Celgene or any of its Affiliates and a Third Party entered into under Section 4.3(d), pursuant to which Celgene or any of its Affiliates in-licenses any Know-How, Materials or Patents that directly relate to or Cover the Elected Candidate and/or Licensed Product or its Manufacture or use.

1.12 "Celgene Regulatory Rights" means all Regulatory Data, Regulatory Filings and Regulatory Approvals for Elected Candidate and Licensed Product worldwide Controlled by Celgene or any of its Affiliates.

1.13 "Celgene Technology" means all Celgene Solely Owned IP and all of Celgene's right, title and interest in and to Joint IP.

1.14 "Clinical Study" means any human clinical trial of a Product Candidate.

1.15 "Commercialization" means any and all activities directed to the Manufacturing, marketing, detailing, promotion and securing of reimbursement of a product after Regulatory Approval has been obtained (including making, having made, using, importing, selling and offering for sale such product), and will include post-approval clinical studies, post-launch marketing, promoting, detailing, marketing research, distributing, customer service, administering and commercially selling such product, importing, exporting or transporting such product for commercial sale, and all regulatory compliance with respect to the foregoing.

1.16 "Commercially Reasonable Efforts" means, with respect to the Development or Commercialization of Licensed Product by a Party, that level of efforts and resources that such Party would normally devote to the Development or Commercialization, as the case may be, of a product owned by it or to which it has rights of the type it has hereunder, which is of a similar commercial potential at a similar stage in its lifecycle, in each case taking into account issues of safety and efficacy, product profile, the proprietary position, the then current competitive environment for such product and the likely timing of such product's entry into the market, the pricing and launching strategy for the respective product, the regulatory environment and status of such product, and other relevant scientific, technical and commercial factors.

1.17 "Control" or "Controlled" means, with respect to any Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals, the possession (whether by ownership or license, other than by a license or sublicense granted pursuant to this License Agreement) by a Party or its Affiliates of the ability to grant to the other Party a license or access as provided herein to such item, without violating the terms of any agreement or other arrangement with any Third Party or, other than under Applicable Bluebird In-Licenses, being obligated to pay any royalties or other consideration therefor ("Additional Payments"). For clarity, Other In-Licenses are not "Controlled" for purposes of this License Agreement, unless and only after such Other In-License is converted into an Applicable New In-License pursuant to Section 3.2(b). Notwithstanding the foregoing, as provided in Section 3.2(a), if on or after the Original License Agreement Effective Date

and for such time as the other Party agrees to pay and does in fact pay all Additional Payments with respect to such Party's access or license to any Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals (other than that in-licensed by Bluebird pursuant to an Other In-License), such Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals will be deemed to be included in the definition of "Control".

1.18 "Covers", with reference to (a) a Patent, means that the making, using, selling, offering for sale or importing of a product or practice of a method would infringe a Valid Claim of such Patent in the country in which such activity occurs, and (b) Materials or Know-How, means that the Manufacture, Development or Commercialization of a product incorporates, embodies or otherwise makes use of such Materials or Know-How.

1.19 "EU" means the organization of member states of the European Union as it may be constituted from time to time.

1.20 "Field" means the targeting of the Target Antigen by the use of (a) T-cells expressing a CAR (with or without other engineering to enhance functionality and/or safety), including virus specific genetically modified T-cells expressing a synthetic CAR, and (b) T-cells expressing native antigen receptors or engineered antigen receptors in which the T-cells are genetically modified to enhance their performance, persistence or safety, in each case under (a) and (b) for the treatment, modulation, palliation or prevention of cancer in humans.

1.21 "First Commercial Sale" means the first sale for use or consumption of any Licensed Product in a country after all required Regulatory Approvals for commercial sale of such Licensed Product have been obtained in such country.

1.22 "First Indication" means the first disease condition for which a particular Licensed Product has been approved by a Regulatory Authority.

1.23 "GAAP" means U.S. generally accepted accounting principles or International Financial Reporting Standards, consistently applied, as designated and used by the applicable Party.

1.24 "Gene Editing" means homing endonuclease (HE) and megaTAL gene editing technologies, including HE/megaTAL-mediated homology directed recombination and Bluebird's proprietary DARIC cell signaling technology.

1.25 "In-License Payments" means any amounts paid or payable under any Applicable Bluebird In-License that are incurred by Bluebird solely and directly as a result of the grant of a sublicense thereunder under this License Agreement to Celgene, any of Celgene's contract Third Parties under Section 3.5, or any further Sublicensees of Celgene (including of Celgene's Affiliates that are granted sublicenses) under this License Agreement. Any such payments will include [***] excluding [***].

1.26 "Licensed IP" means all (a) Patents, Materials and Know-How Controlled at any time during the term of this License Agreement by Bluebird or any of its Affiliates (including any applicable Collaboration IP and Bluebird Technology), other than pursuant to an Applicable Bluebird In-License, and (b) Bluebird In-Licensed IP, in each case to the extent necessary or useful to Develop Elected Candidate and Develop and Commercialize Licensed Product. [***].

1.27 "Licensed Product" means any product that constitutes or incorporates an Elected Candidate (including all modified and improved versions thereof), in all forms, presentations, and formulations (including manner of delivery and dosage). A modified or improved version of an

Elected Candidate constituted or incorporated in a product will be deemed a "Modified Licensed Product" for purposes of Section 4.2 if it is Covered by patentable technology Controlled by Bluebird that (a) is first discovered, created, conceived, developed or reduced to practice after the later of (i) the Original License Agreement Effective Date and (ii) the end of the Collaboration Program Term, (b) requires the submission of a new BLA with respect to such modified or improved Elected Candidate, and (c) materially contributes to the Elected Candidate being approved for a new indication or new patient population. For clarity, "Modified Licensed Products" are Licensed Products hereunder for all purposes other than Section 4.2.

1.28 "Manufacturing" means the production, manufacture, processing, filling, finishing, packaging, labeling, shipping and holding of product or any intermediate thereof, including process development, process qualification and validation, scale-up, commercial manufacture and analytic development, product characterization, stability testing, quality assurance and quality control. With reference to Elected Candidate and Licensed Product, Manufacturing includes Vector and associated Payload supply.

1.29 "Net Sales" means with [***].

1.30 "Pivotal Study" means (a) a Phase 3 Study that is intended by Celgene to be submitted (together with any other registration trials that are prospectively planned when such Phase 3 Study is initiated) for Regulatory Approval in the U.S., or (b) any other clinical study that is designed to establish that a pharmaceutical product is safe and efficacious for its intended use, and to determine warnings, precautions, and adverse reactions that are associated with such pharmaceutical product in the dosage range to be prescribed, which clinical study is a registration trial intended to be sufficient for filing an application for a Regulatory Approval for the Licensed Product in the U.S., solely as evidenced by the acceptance for filing for a Regulatory Approval for such product after completion of such study.

1.31 "Regulatory Exclusivity Period" means with respect to a Licensed Product in a country, the period of time during which (a) Celgene or any of its Affiliates or Sublicensees has been granted the exclusive legal right by a Regulatory Authority (or is otherwise entitled to the exclusive legal right by operation of Law) in such country to market and sell the Licensed Product, or (b) the data and information submitted by Celgene or any of its Affiliates or Sublicensees to the relevant Regulatory Authority in such country for purposes of obtaining Regulatory Approval may not be disclosed, referenced or relied upon in any way by such Regulatory Authority (including by relying upon the Regulatory Authority's previous findings regarding the safety or effectiveness of the Licensed Product) to support the Regulatory Approval or marketing of any product by a Third Party in such country.

1.32 "ROW" means the world other than the United States.

1.33 "ROW Administration" means administration of Licensed Product to a patient when located in the ROW.

1.34 "Second Indication" means a [***].

1.35 "Selling Party" means Celgene and its Sublicensees (including Celgene's Affiliates that are granted sublicenses pursuant to Section 3.3).

1.36 "Sublicensee" means any person or entity (including Affiliates of Celgene) that is granted a sublicense as permitted by Section 3.3 (or an option to take such a sublicense), either directly by Celgene or indirectly by any other Sublicensee hereunder.

1.37 "Target Antigen" means the antigen designated as B-cell maturation antigen (BCMA) as further set forth on Appendix D, and naturally occurring variants thereof.

1.38 "U.S. Administration" means administration of Licensed Product to a patient when located in the United States.

1.39 "Valid Claim" means, with respect to a particular country, (a) any claim of an issued and unexpired Patent in such country that (i) has not been held revoked, unenforceable or invalid by a decision of a court or governmental agency of competent jurisdiction, which decision is unappealable or unappealed within the time allowed for appeal and (ii) has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue or disclaimer or otherwise in such country, or (b) a claim of a pending Patent application that has not been finally abandoned or finally rejected or expired and which has been pending [***] from the date of filing of the earliest priority Patent application to which such pending Patent application is entitled to claim benefit.

1.40 "Vector" means recombinant lentiviral agent(s) (including all components therein other than Payloads) for gene therapy intended to deliver a nucleotide sequence, including those recombinant viral agent(s) (including all components therein other than Payloads) for any Elected Candidate or Licensed Product and Manufactured utilizing the [***] under this Agreement. For avoidance of doubt, Vectors do not include Payloads.

1.41 "Vector Supplies" means supplies of Vectors and associated Payloads Manufactured for incorporation into Elected Candidate and Licensed Product for Development or Commercialization thereof.

Definitions for each of the following terms are found in the body of this License Agreement or the Appendices hereto as indicated below:

Defined Terms	***Location***
Additional IP	Section 3.2(a)
Additional Payments	Section 1.17
Applicable Bluebird In-License	Section 1.1
Applicable New In-License	Section 1.2
Applicable Pre-Existing In-License	Section 1.3
Bankruptcy Code	Section 3.7
Bioreliance	Section 2.4(b)(ii)(B)
Biosimilar Application	Section 7.2(f)
Biosilimar Product	Section 1.4
Biosimilar Product Competition	Section 4.3(e)
Bluebird	Preamble
Bluebird In-Licensed IP	Section 1.5
Bluebird Indemnitees	Section 9.6(a)
Bluebird Technology	Section 1.6
Celgene	Preamble
Celgene Corp	Preamble
Celgene Development & Commercialization Program	Section 1.7
Celgene Europe	Preamble
Celgene Indemnitees	Section 9.6(b)
Celgene Licensed Product In-License	Section 1.8

Defined Terms	***Location***
Celgene Licensed Product In-Licensed IP	Section 1.9
Celgene Licensed Product IP	Section 1.10
Celgene Other In-License	Section 1.11
Celgene Regulatory Rights	Section 1.12
Celgene Technology	Section 1.13
Clinical Data	Section 8
Clinical Study	Section 1.14
Combination Product	Section 1.29
Commercialization	Section 1.15
Commercially Reasonable Efforts	Section 1.16
Competitive Infringement	Section 7.1
Control	Section 1.17
Covers	Section 1.18
Elected Candidate	Appendix A
EU	Section 1.19
Eurogentec	Section 2.4(b)(ii)(B)
Field	Section 1.20
First Commercial Sale	Section 1.21
First Indication	Section 1.22
Fully Burdened Manufacturing Cost	Appendix H
GAAP	Section 1.23
Gene Editing	Section 1.24
Independent Target Antigen Program	Section 3.4
In-License Payment	Section 1.25
Indemnification Claim Notice	Section 9.6(c)
Indemnified Party	Section 9.6(c)
Joint IP	Section 5.2
License Agreement	Preamble
License Agreement Term	Section 10.1
Licensed IP	Section 1.26
Licensed Product	Section 1.27
Litigation Conditions	Section 9.6(d)(i)
Losses	Section 9.6(a)
Manufacturing	Section 1.28
Manufacturing Party	2.4(b)(i)(E)
Manufacturing and Supply Agreement	Section 2.4(b)(i)(B)
Master Collaboration Agreement	Preamble
Milestone Event	Section 4.2
Milestone Payment	Section 4.2
Modified Licensed Product	Section 1.27
Net Sales	Section 1.29
Original MCA	Preamble
Original License Agreement Effective Date	Preamble

Defined Terms	***Location***
Party(ies)	Preamble
Patent Challenge	Section 10.2(b)
PHSA	Section 7.2(f)
Pivotal Study	Section 1.30
Regulatory Exclusivity Period	Section 1.31
Second Indication	Section 1.32
Selling Party	Section 1.35
Solely Owned IP	Section 5.1
Specific Patent	Section 6.3
Sublicensee	Section 1.36
Suspension Transition Plan	Section 2.4(b)(i)(A)
Third Party Claims	Section 9.6(a)
Valid Claim	Section 1.38
Vector Supplies	Section 1.40

2. Development and Commercialization.

2.1 Development. As of and after the Original License Agreement Effective Date, Celgene will assume sole responsibility for, and control of, Developing Elected Candidate and Licensed Product in the Field worldwide, and will establish a Celgene Development & Commercialization Program for that purpose. As of and after the Original License Agreement Effective Date, Celgene will have sole responsibility for all costs and expenses arising from the Development and Commercialization of Elected Candidate and Licensed Product in the Field worldwide. Notwithstanding the foregoing, if the initial Phase 1 Study with respect to Optioned Candidate has not been completed as of the Original License Agreement Effective Date, Bluebird will continue to be responsible for the performance of such initial Phase 1 Study under the oversight of the JSC under the Master Collaboration Agreement until completion of such initial Phase 1 Study. In the event Bluebird continues to be responsible for the performance of such initial Phase 1 Study, Bluebird will be responsible for the costs of performing such initial Phase 1 Study on the terms set forth in the Master Collaboration Agreement.

2.2 Regulatory. Subject to the last sentence of Section 2.1, (a) as of and after the Original License Agreement Effective Date, Celgene will lead and have sole control of all efforts with Regulatory Authorities regarding the Development and Commercialization of Elected Candidate and Licensed Product in the Field worldwide, including taking full responsibility for preparing and filing the relevant Regulatory Filings and seeking Regulatory Approval and (b) promptly following the Original License Agreement Effective Date, Bluebird will, at Celgene's expense, assign to Celgene all Regulatory Filings with respect to Elected Candidate and Licensed Product. For clarity, in the event Bluebird continues to be responsible for the performance of an initial Phase 1 Study following the Original License Agreement Effective Date in accordance with Section 2.1, Bluebird will retain ownership of any Regulatory Filings (including the IND) for Optioned Candidate until completion of such initial Phase 1 Study. In the event of failure to assign such Regulatory Filings to Celgene, Bluebird hereby consents and grants to Celgene the right to access and reference (without any further action required on the part of Bluebird, whose authorization to file this consent with any Regulatory Authority is hereby granted) any such Regulatory Filing.

2.3 Technical Assistance. During the Collaboration Program Term, Bluebird will reasonably cooperate with Celgene to provide all technical assistance, and to transfer to Celgene any additional Know-How licensed to Celgene under Section 3.1, requested by Celgene to facilitate the transfer of Development efforts related to Elected Candidate and Licensed Product. Such cooperation will include providing Celgene with reasonable access by teleconference or in-person at Bluebird's facilities to Bluebird personnel involved in the research and Development of Elected Candidate to provide Celgene with a reasonable level of technical assistance and consultation in connection with the transfer of such Know-How. Following the Collaboration Program Term, Bluebird will reasonably cooperate with Celgene to provide reasonable amounts of technical assistance, including to transfer to Celgene any additional Know-How licensed to Celgene under Section 3.1, with respect to Elected Candidate or Licensed Product as reasonably requested by Celgene with reasonable advance notice to Bluebird. Any dispute with respect to the amount and completeness of the technical assistance and cooperation to be provided by Bluebird under this Section 2.3 will be referred to and finally resolved by binding arbitration by a mutually agreeable, disinterested, conflict-of-interest-free individual not affiliated or consulting with either Party. Any such arbitration will be conducted under the then-current rules of the American Arbitration Association.

2.4 Manufacture and Supply.

(a) *Manufacturing Generally*. Subject to Section 2.4(b), Celgene will be solely responsible for, and will bear all the costs and expenses of, Manufacturing and supplying all Elected Candidate and Licensed Product for Development and Commercialization in the Field worldwide. Subject to Section 2.4(b), Celgene will purchase Vector Supply from Bluebird or its authorized designee for such purposes. Notwithstanding anything herein to the contrary, subject to, and with effect from, the expiry or termination of the Manufacturing and Supply Agreement, Celgene will assume sole responsibility for the Manufacture and supply of Vector including associated Payloads for the Development and Commercialization of Elected Candidate and Licensed Product for U.S. Administration and ROW Administration in accordance with this License Agreement.

(b) *Vector Manufacturing*.

(i) Vector Supply Terms.

(A) Bluebird shall use Commercially Reasonable Efforts to qualify its manufacturing facility for the Manufacture of Vector. Unless otherwise agreed by the Parties in writing, within [***] the Parties will negotiate in good faith a transfer plan to be agreed by the Parties, to engage in a technology transfer as set forth in Section 2.4(b)(i)(E)(the "**Suspension Transition Plan**"). The Parties will use Commercially Reasonable Efforts to finalize the Suspension Transition Plan within [***]. The Parties shall commence the technology transfer activities referred to in such Suspension Transition Plan within [***]. From the date of U.S. approval of Bluebird's facility for Vector and until completion of the Suspension Transition Plan and subject to the terms and conditions of the Manufacturing and Supply Agreement, Bluebird shall solely be responsible for the Manufacture of Vector and associated Payloads for U.S. Administration and ROW Administration. After completion of the Suspension Transition Plan, Bluebird and its Affiliates will be primarily responsible for the Manufacture of Vector and associated Payloads for all Elected Candidate and Licensed Product required for clinical Development and Commercialization in the Field for U.S. Administration, and Bluebird will collaborate in good faith and use Commercially Reasonable Efforts to be Celgene's secondary source of supply for the Manufacture of Vector and associated Payloads for Elected Candidate and Licensed Product required for clinical Development

and Commercialization in the Field for ROW Administration in each case, solely in connection with such "back-up" or "business continuity source" rights under the Manufacturing and Supply Agreement.

(B) The Parties will enter into a "Manufacturing and Supply Agreement," between each other or among the Parties and an Affiliate, covering Vector Supply and associated Payloads within [***] which agreement will be consistent with the terms of this Section 2.4(b)(i) and will otherwise be subject in all respects to the terms and conditions of this License Agreement (the "**Manufacturing and Supply Agreement**").

(C) The cost to Celgene of Vector Supply will equal [***] of Bluebird's Fully Burdened Manufacturing Cost for such Manufacture, plus [***] unless otherwise agreed by the Parties in writing.

(D) The Manufacturing and Supply Agreement will include the terms set forth in Appendix I, including license grants from Celgene to Bluebird under the Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP to the extent necessary or useful for Bluebird to Manufacture Vector Supply.

(E) In accordance with Appendix I, Bluebird will use Commercially Reasonable Efforts, to engage in a technology transfer to allow Celgene, in accordance with Section 2.4(b)(i), to Manufacture Vector (through the first commercial batch of Vector) itself or by through its designated Third Party manufacturer (each a "**Manufacturing Party**"), by transferring all Know-How and Materials Controlled by Bluebird or its Affiliates that are necessary to Manufacture Vector. Celgene shall bear [***] and Bluebird shall bear [***] of the Costs and expenses of the Parties associated with such technology transfer. Notwithstanding the foregoing, Bluebird shall only be required to deliver Know-How and Materials in its or its Affiliates' actual possession or under its control and shall not be required to produce or create any additional Know-How or Materials. Before any such transfer, the Manufacturing Party shall enter into a reasonable confidentiality agreement with Bluebird with respect to the use and handling of such Know-How and Materials.

(F) Celgene will use Commercially Reasonable Efforts to establish a second source of Vector within [***].

(G) Any purchase of Vector Supply from Bluebird or its designee will expressly not include any license rights to any Know-How or Patents, but instead all licenses (implied, by exhaustion or otherwise) will arise under Section 3.1, if and as applicable.

(H) For the purpose of this License Agreement, certain words and phrases (and their correlatives) relating to Manufacturing will have the meanings set forth on Appendix I.

(I) Celgene agrees to collaborate in good faith with Bluebird and use Commercially Reasonable Efforts to Manufacture Vector for U.S. Administration to the extent circumstances would require Bluebird to activate "business continuity source" supply for U.S. Administration. Bluebird agrees to collaborate in good faith with Celgene and use Commercially Reasonable Efforts to Manufacture Vector for ROW Administration to the extent circumstances would require Bluebird to activate "business continuity source" supply for ROW Administration pursuant to the Manufacturing and Supply Agreement.

(J) For as long as Bluebird is sole source of supply of Vector, in the event of any supply deficiency or shortage of Vector or associated Payload, any available Vector or Payload supplies shall be allocated for U.S Administration and ROW Administration on pro rata basis, using

the forecasted demand for the year in which such deficiency or shortage occurs, unless otherwise agreed by the Parties in writing.

(ii) Payloads.

(A) Celgene shall have the right to conduct quality audits of Bluebird's existing inventories of Bluebird's of [***] and shall have the right to purchase from Bluebird, [***] with sufficient shelf life and in sufficient quantities to allow Celgene to Manufacture Vector in accordance with this License Agreement while Celgene establishes the supply arrangements referred to in Section 2.4(b)(ii)(B).

(B) Bluebird will take such actions as are necessary to permit Celgene to purchase quantities of [***] solely for use in Manufacturing Vector for Elected Candidate and Licensed Products as permitted under this License Agreement, under and pursuant to a supply or similar agreement between Celgene and [***] respectively, and Bluebird will execute and deliver a letter of authorization or similar document to [***] respectively, to authorize such purchases. Forecasting for plasmids will be reviewed and approved by the Parties on a quarterly basis. Information received from [***] relating to the plasmids sequence shall be deemed to be Bluebird's Confidential Information for purposes of this License Agreement. In addition, Bluebird will take such actions as are necessary to permit Celgene to purchase quantities of [***] for use in Manufacturing Vector for Elected Candidate and Licensed Products as permitted under this License Agreement, under and pursuant to a supply or similar agreement between Celgene and [***] and, to the extent required to enable such purchases, Bluebird will execute and deliver a letter of authorization or similar document to [***].

2.5 Celgene Diligence. Celgene, directly or through one or more of its Sublicensees, will use Commercially Reasonable Efforts: (a) to Develop Licensed Product in the Field and to obtain Regulatory Approvals therefor; and (b) to Commercialize Licensed Product in the Field after obtaining such Regulatory Approval, in each country worldwide where Regulatory Approval has been obtained. With respect to the aforementioned obligation to use Commercially Reasonable Efforts in relation to Licensed Product for ROW Administration, Celgene shall be required to use such Commercially Reasonable Efforts solely to the extent necessary to enable Bluebird to comply with the Applicable Bluebird-In Licenses.

2.6 Annual Update Meetings. At least once during each consecutive twelve (12)-month period from the Original License Agreement Effective Date until the earlier of first approval of a BLA for Licensed Product by the FDA, within [***] of Bluebird's written request, the Parties will meet in person at a U.S. site of Celgene for Celgene to provide Bluebird with an update on the Development of Licensed Product by Celgene and its Sublicensees for U.S. Administration. During such meeting, Celgene will disclose to Bluebird all material information regarding such Development.

2.7 Reports by Celgene. Celgene will prepare and maintain, and will cause its Sublicensees to prepare and maintain, reasonably complete and accurate records regarding the Development of Elected Candidate and Licensed Product, and Commercialization of Licensed Product worldwide after Regulatory Approval therefor. Celgene will provide to Bluebird a reasonably detailed report regarding such efforts at least once every twelve (12)-month period from the Original License Agreement Effective Date. In relation to Licensed Product for U.S. Administration, such report will contain sufficient detail to enable Bluebird to assess Celgene's compliance with its Development and Commercialization obligations in Section 2.5, including

information with respect to the following: (a) the design, status and results of any animal studies and clinical trials for Licensed Product; (b) any regulatory milestones, and any Regulatory Approvals achieved, for Licensed Product; and (c) activities with respect to selling, promoting, supporting, detailing and marketing of Licensed Product. In addition to the foregoing, Celgene will provide Bluebird with such additional information regarding any such activities as Bluebird may reasonably request from time to time. In relation to Licensed Product for ROW Administration, such report will contain sufficient detail to enable Bluebird to comply with the Applicable Bluebird In-Licenses. In addition to the foregoing, Celgene will provide Bluebird with such additional information regarding any such activities as Bluebird may reasonably request from time to time to the extent reasonably necessary to enable Bluebird to comply with Applicable Bluebird In-Licenses.

2.8 Applicable Bluebird In-Licenses and Other IP.

(a) *Maintenance of Applicable Bluebird In-Licenses*. Bluebird (i) will duly perform and observe all of its obligations under the Applicable Bluebird In-Licenses in all material respects and maintain in full force and effect the Applicable Bluebird In-Licenses, and (ii) will not, without Celgene's prior written consent (such consent not to be unreasonably withheld, conditioned or delayed), [***]. Bluebird will provide Celgene with written notice as promptly as practicable (and in any event within [***] business days) after becoming aware of any of the following: [***]. If Bluebird fails to pay any amounts due under any Applicable Bluebird In-License [***] Celgene will have the right, but not the obligation, in its sole discretion, to [***].

(b) *Maintenance of Celgene Licensed Product In-Licenses*. Celgene (i) will duly perform and observe all of its obligations under the Celgene Licensed Product In-Licenses in all material respects and maintain in full force and effect the Celgene Licensed Product In-Licenses, and (ii) will not, without Bluebird's prior written consent (such consent not to be unreasonably withheld, conditioned or delayed), [***]. Celgene will provide Bluebird with written notice as promptly as practicable (and in any event within [***] business days) after becoming aware of any of the following: [***]. If Celgene fails to pay any amounts due under any Celgene Licensed Product In-License and [***] Bluebird will have the right, but not the obligation, in its sole discretion, to [***].

(c) *Applicable Bluebird In-License Requirements*. Celgene will abide, and will cause all its Affiliates and applicable Sublicensees to abide, by all requirements of each Applicable Bluebird In-License in all material respects (and in any case in all respects in the case that failure to so abide would result in a breach under the Applicable Bluebird In-License), to the extent applicable to Sublicensees thereunder and to the extent disclosed by Bluebird to Celgene, with the understanding that disclosure by Bluebird of any Applicable Bluebird In-License to Celgene will be deemed disclosure of such requirements of such Applicable Bluebird In-License to Celgene. In the event of a termination of any Applicable Bluebird In-License, Bluebird agrees, to the extent requested by Celgene, to reasonably assist Celgene in securing a direct license from the applicable licensor under any Patents, Materials and Know-How that was licensed to Bluebird and sublicensed to Celgene hereunder prior to such termination. In addition, Bluebird agrees, if requested by Celgene, to reasonably assist Celgene in securing a standby license from the applicable licensor under any Patents, Materials and Know-How that are licensed to Bluebird and sublicensed to Celgene.

3. License Grants.

3.1 License by Bluebird. Subject to the terms and conditions of this License Agreement, Bluebird hereby grants to Celgene a worldwide, exclusive (even as to Bluebird except as set forth in

Section 2.4(b), license, with the right to sublicense only as permitted by Section 3.4, under Licensed IP, to Develop Elected Candidate and to Develop and Commercialize Licensed Product. Further, (a) the license to Commercialize granted in this Section 3.1 will cover only the sale and offer for sale of Licensed Product in finished form and not the sale or offer for sale of Vectors or Payloads (other than as and to the extent incorporated in the Licensed Product), and (b) rights to Manufacture Vectors and associated Payloads are included within the scope of the license granted to Celgene under this Section 3.1, which rights are subject to the terms and conditions of Section 2.4(b)(i). Celgene's right to Manufacture Vector Supply pursuant to this Section 3.1 will be exercised by Celgene solely (i) for Development and Commercialization for ROW Administration after the technology transfer set forth in Section 2.4(b)(i)] , and (ii) for Development and Commercialization for U.S. Administration, solely in connection with such "back-up" and/or "second source" rights under the Manufacturing and Supply Agreement, and Celgene will not otherwise exercise the license to Manufacture Vector Supply set forth in Section 3.1. Notwithstanding the foregoing, subject to, and with effect from, the expiry or termination of the Manufacturing and Supply Agreement, Celgene will assume sole responsibility for the Manufacture of Vector for Development and Commercialization of Elected Candidate and Licensed Product for U.S. Administration and ROW Administration in accordance with this CCPS Agreement.

3.2 Additional IP; Other In-Licenses.

(a) *Additional IP*. Except as set forth in Section 3.2(b), Celgene may, on or after the Original License Agreement Effective Date, elect to include within the scope of the Licensed IP any Know-How, Material, Patent, Regulatory Data, Regulatory Filings or Regulatory Approvals ("Additional IP"), that would be Controlled by Bluebird but for required payments of Additional Payments to a Third Party, by (i) providing notice to Bluebird of same and (ii) agreeing to pay and in fact paying all Additional Payments with respect to Celgene's access or license to such Additional IP. Following Bluebird's receipt of such notice and subject to Celgene's performance of its obligations to pay any Additional Payments with respect to Celgene's access or license to such Additional IP, such Additional IP will be deemed Licensed IP hereunder. For avoidance of doubt, this Section 3.2(a) does not apply to Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals licensed to Bluebird under the Applicable Bluebird In-Licenses, all of which are deemed Controlled by Bluebird notwithstanding this Section 3.2(a).

(b) *Other In-Licenses*. Celgene may, on or after the Original License Agreement Effective Date, elect to convert any Other In-License to an Applicable New In-License by providing notice to Bluebird of same. Upon Bluebird's receipt of such notice, such Other In-License will be an Applicable New In-License hereunder, Appendix B will automatically be updated to include such New In-License and the provisions of this License Agreement applicable to New In-Licenses, including Section 4.1(b), will apply with respect to such New In-License.

3.3 Sublicensing Rights.

(a) *Transfer*. The licenses granted in Sections 3.1 are transferable only upon a permitted assignment of this License Agreement in accordance with Section 11.12.

(b) *Celgene Sublicenses*. The license granted in Section 3.1 may be sublicensed, in full or in part, by Celgene by a written agreement to its Affiliates and Third Parties (with the right to sublicense through multiple tiers), provided, that as a condition precedent to and requirement of any such sublicense:

(i) Celgene will provide Bluebird with a copy of any sublicense agreement with a non-Affiliated Sublicensee within [***] of execution thereof, and to the extent permitted under any Applicable Bluebird In-License, such sublicense agreement may be redacted as necessary to protect commercially sensitive information;

(ii) Celgene will be responsible for any and all obligations of such Sublicensee as if such Sublicensee were "Celgene" hereunder; and

(iii) Any such Sublicensee will agree in writing to be bound by substantially identical obligations as Celgene hereunder with respect to the activities of such Sublicensee hereunder (and not with respect to the activities of any other), including Know-How disclosure obligations Celgene has to Bluebird hereunder with respect to the activities of such Sublicensee hereunder (but excluding payment obligations).

3.4 Exclusivity.

(a) Each Party and its Each Party and its Affiliates may research, Develop, Manufacture or Commercialize any actual or potential products (other than Elected Candidate, Licensed Product orbb2121) to be used in the Field (which, for the purposes of this Section 3.4(a), will include all indications and will not be limited to cancer) that specifically target the Target Antigen internally or with Third Party collaborators, licensors, licensees or partners (any such program, an "**Independent Target Antigen Program**"), provided that (A) in the case of Bluebird, (i) none of the Celgene Licensed Product In-Licensed IP and none of the Celgene Licensed Product IP , or other Patents, Materials or Know-How Controlled by Celgene and licensed to Bluebird hereunder will be used by Bluebird in the conduct of its Independent Target Antigen Programs, (ii) subject to Article 8, none of the Confidential Information of Celgene will be used by Bluebird in its conduct of Independent Target Antigen Programs, and (iii) Bluebird will have appropriate internal procedures in place to ensure compliance with provisos (i) and (ii) of this clause (A) and (B) in the case of Celgene, (i) none of the Licensed IP, or other Patents, Materials or Know-How Controlled by Bluebird and licensed to the Celgene hereunder will be used by Celgene in the conduct of its Independent Target Antigen Programs, (ii) subject to Article 8, none of the Confidential Information of Bluebird will be used by Celgene in its conduct of Independent Target Antigen Programs, and (iii) Celgene will have appropriate internal procedures in place to ensure compliance with provisos (i) and (ii) of this clause (B).

3.5 Contract Manufacturers. Subject to the terms and conditions of this License Agreement, Celgene will have the right to appoint by a written agreement "contract manufacturers", meaning any Third Party or Affiliate of Celgene that Manufactures Licensed Product (or components therefor, including Vectors and associated Payloads) for re-sale, but who itself is not a "Sublicensee" hereunder and thereby exercises "have made" rights granted by the other Party hereunder. Subject to the terms and conditions of this License Agreement, Celgene will have the right to appoint by a written agreement "contract research organizations" and other providers performing services on Celgene's behalf, none of which will be deemed a "Sublicensee" hereunder. Celgene will be responsible for any such contract manufacturer, contract research organization or service provider hereunder, and further will require any such contract manufacturer, contract research organization or service provider to agree in writing to comply with Sections 3.6 and 8. Celgene shall have the right to audit any Third Party contract manufacturer engaged by Bluebird, including in relation to the Manufacture of Vector for supply to Celgene pursuant to the Manufacturing and Supply Agreement. Notwithstanding the foregoing, if, at any time, Bluebird determines that it is appropriate or desirable

to outsource the Manufacture of the Vector for U.S. Administration to a Third Party, and provided that Celgene has filed for U.S. approval of a second source of supply of Vector, Bluebird shall notify Celgene in writing and shall, before engaging into any request for proposal or similar procurement process, consult with Celgene regarding possible options for obtaining such supply, which may include having Celgene or one of its Affiliates become solely responsible for the Manufacture Vector Supply of U.S. Administration. In the event that Bluebird, after such consultation, determines to engage an alternative or additional manufacturer for the Manufacture of the Vector for U.S. Administration, Celgene and its Affiliates shall have the right (but not the obligation) to bid in this process in accordance with the bid procedures made available by Bluebird. If Bluebird receives a bona fide offer from a Third Party manufacturer reasonably acceptable to Celgene from a quality and creditworthiness perspective, Celgene shall have the right to meet or exceed such Third Party' offer and become the selected manufacturer. In any event, Bluebird shall not enter into any agreement with the selected Third Party manufacturer without Celgene's prior written consent, which will not be unreasonably withheld, conditioned or delayed.

3.6 No Implied Rights. No license, sublicense or other right is or will be created or granted hereunder by implication, estoppel or otherwise. Any licenses, sublicenses or rights will be granted only as expressly provided in this License Agreement. Celgene will not practice or otherwise use any Licensed IP other than in accordance with the licenses granted in Section 3.1.

3.7 Section 365(n) of the Bankruptcy Code. All rights and licenses granted pursuant to any section of this License Agreement are, and will be deemed to be, rights and licenses to "intellectual property" (as defined in Section 101(35A) of title 11 of the United States Code and of any similar provisions of applicable Laws under any other jurisdiction (the "Bankruptcy Code")). Bluebird agrees that Celgene, as a licensee of rights and licenses under this License Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against Bluebird under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, Celgene will be entitled to a complete duplicate of (or complete access to, as appropriate) any intellectual property licensed to Celgene and all embodiments of such intellectual property, which, if not already in Celgene's possession, will be promptly delivered to it (a) upon any such commencement of a bankruptcy proceeding upon Celgene's written request therefor, unless Bluebird elects to continue to perform all of its obligations under this License Agreement or (b) if not delivered under clause (a), following the rejection of this License Agreement by Bluebird in the bankruptcy proceeding upon written request therefor by Celgene.

4. Payments and Royalties.

4.1 Applicable Bluebird In-Licenses and Celgene Licensed Product In-Licenses.

(a) *Applicable Pre-Existing In-Licenses*. If any In-License Payment becomes due under any Applicable Pre-Existing In-License during the License Agreement Term, Bluebird will pay same, provided that Celgene will reimburse Bluebird for any such In-License Payment within thirty (30) days of Celgene's receipt of Bluebird's written invoice therefor, which In-License Payment (other than payments that are royalties) will not exceed [***] and subject to Section 6.1. Any such reimbursement by Celgene to Bluebird (i) is in addition to and not in lieu of the other payments required by this Section 4 and (ii) will not be subject to Section 4.3(d).

(b) *Applicable New In-Licenses*. Celgene may elect to take a sublicense under any New In-License of Bluebird and its Affiliates and upon such election, such New In-License will be an Applicable New In-License hereunder for all purposes. For the purposes of determining the Parties' respective payment obligations, all Applicable New In-Licenses as of and following the Original License Agreement Effective Date will be listed on Appendix B. If any In-License Payment becomes due under any Applicable New In-License during the License Agreement Term, Bluebird will pay same and, subject to Section 6.1, Celgene will reimburse Bluebird for (i) [***] of such payment that are royalties, and (iii) [***] of such payment that are not royalties, in each case ((i) and (ii)) within thirty (30) days of receipt of Bluebird's written invoice therefor. If Celgene elects to convert an Other In-License to an Applicable New In-License pursuant to Section 3.2(b), Celgene will reimburse Bluebird for [***] of any In-License Payments that became due under such Applicable New In-License during the License Agreement Term to the same extent as if such Applicable New In-License was designated as such as of the Original License Agreement Effective Date, including with respect to applicable Patent Costs in accordance with Section 6.1, provided that Bluebird provides Celgene with a reasonable accounting of same. If any In-License Payments are royalties due under any Applicable New In-License during the License Agreement Term that directly relate to the Commercialization of the Elected Candidate and Licensed Product in the United States, such royalties will be subject to Section 4.3(d). To the extent that any grant of a sublicense by Celgene or any Sublicensees under an Applicable New In-License triggers a payment obligation under such Applicable New In-License, Bluebird will pay same and Celgene will reimburse Bluebird for [***] of such payment within thirty (30) days of receipt of Bluebird's written invoice therefor.

(c) *Celgene Licensed Product In-Licenses*. If any payments become due under any Celgene Licensed Product In-License with respect to the Licensed Product, Bluebird will be responsible for [***] such payments as provided in Section 4.1(e) of the Master Collaboration Agreement, provided that if any such payments are royalties for U.S. Administration, such royalties will be subject to Section 4.3(d).

4.2 Milestone Payments. Celgene will make milestone payments (each, a "Milestone Payment") to Bluebird upon the occurrence of each of the milestone events (each, a "Milestone Event") as set forth below in this Section 4.2. Each of the Milestone Payments will be payable to Bluebird by Celgene within forty-five (45) days of the achievement of the specified Milestone Event, and such payments when owed or paid will be non-refundable and non-creditable, and not subject to set-off, except as otherwise set forth in Sections 2.8(a), 10.3(c) and 10.6 hereof, and Sections 4.1(e), 4.3 and 10.6 of the Master Collaboration Agreement. Except with respect to Modified Licensed Products, each of the Milestone Payments are payable only once in total under this License Agreement, whether achieved by one or more Licensed Products. Notwithstanding the foregoing, Bluebird will be entitled to receive [***] of the Milestone Payments below, other than the Milestone Payment for the first Milestone Event [***].

Milestone Event	***Milestone Payment***
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

*[***].

4.3 Royalties.

(a) *Rates*. Subject to the remainder of this Section 4.3, Celgene will pay to Bluebird running royalties, on a Licensed Product-by-Licensed Product basis, based on the total aggregate annual Net Sales in the United States by Selling Parties of such Licensed Product in a given calendar year at the following royalty rates:

Annual Net Sales in the U.S. of each Licensed Product	***Royalty Rate***
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

By way of example, in a given calendar year, if the aggregate annual Net Sales in the United States for a Licensed Product is [***] the following royalty payment would be payable for those Net Sales under this Section 4.3(a): [***].

(b) *Royalty Term*. Royalties under Section 4.3(a) will be payable, on a Licensed Product-by-Licensed Product, on the Net Sales of any Licensed Product in the United States if at least one of the following two (2) conditions apply:

(i) if one or more Valid Claims within any of Patents included within the Licensed IP (including, for clarity, Joint IP) Covers such Licensed Product in the United States; or

(ii) for [***] from the First Commercial Sale of such Licensed Product in the United States, provided that, for the purposes of this Section 4.3(b)(ii), Licensed Products that have achieved Regulatory Approval under different BLAs will be deemed to be separate Licensed Products hereunder, and thus subject to separate [***] periods.

(c) *Royalty Reduction*. If Licensed Product is royalty-bearing only on account of Section 4.3(b)(ii), then the royalty rates set forth in Section 4.3(a) with respect to Net Sales attributable to Licensed Product will be reduced by [***].

(d) *Third Party Royalty Payments*. If Celgene or its Sublicensee, in its reasonable judgment, is required to obtain a license from any Third Party under any Patent Covering Licensed Product in order to Develop or Commercialize such Licensed Product in the United States, and if Celgene (or its Sublicensee) is required to pay to such Third Party under such license any royalties, and the infringement of such Patent cannot reasonably be avoided by Celgene (or its Sublicensee), or if Celgene (or its Sublicensee) is required by a court of competent jurisdiction to pay royalties or lost profits to such a Third Party (and the infringement of such Patent cannot reasonably be avoided), then the amount of Celgene's royalty obligations under this Section 4.3 will be reduced by [***] of the amount of such royalties paid to such Third Party, provided however, that the royalties payable under Section 4.3(a) will not be reduced in any such event below [***] of the amounts set forth in Section 4.3(a) (but as may be further reduced pursuant to Section 4.3(c) or Section 4.3(e)) for each royalty tier. Any royalties payable under any Applicable Pre-Existing In-Licenses that directly relate to the Commercialization of the Elected Candidate or Licensed Product in the United States may not be deducted under this Section 4.3(d) from royalties owed to Bluebird. Any royalties payable under any Applicable New In-Licenses and Celgene Licensed Product In-Licenses may be deducted under this Section 4.3(d) from royalties owed to Bluebird. Celgene (or its Sublicensee) will use its

commercially reasonable efforts to minimize the amount of any of the foregoing payments owed to Third Parties. Prior to Celgene or its Sublicensee exercising its reasonable judgment under this Section 4.3(d), Celgene will provide Bluebird with written notice of a potential need to obtain any license from Third Parties. The Parties will discuss the best course of action to resolve such potential license requirement(s).

(e) [***].

(f) *Additional Royalty Provisions*. The royalties payable under Section 4.3(a) will be subject to the following:

(i) only one (1) royalty will be payable hereunder with respect to each Licensed Product unit;

(ii) royalties when owed or paid hereunder will, except as provided in Section 4.3(d), be non-refundable and non-creditable and not subject to set-off (except as otherwise provided in Sections 2.8(a), 10.3(c) and 10.6 hereof, Section 17.6 of any Co-Development, Co-Promote and Profit Share Agreement, and Sections 4.1(e), 4.3 and 10.6 of the Master Collaboration Agreement); and

(iii) except as expressly set forth in Sections 4.3(c), 4.3(d) and 4.3(e), no other royalty deductions are permitted hereunder.

4.4 Payment Terms.

(a) *Manner of Payment*. All payments to be made by Celgene hereunder will be made in U.S. dollars by wire transfer to such bank account as Bluebird may designate.

(b) *Reports and Royalty Payments*. For as long as royalties or other payments are due under this Section 4, Celgene will furnish to Bluebird a written report, after the end of each calendar quarter, showing the amount of Net Sales and royalty due under Section 4.3, and any other payments accrued during such calendar quarter, which report will be furnished within [***] of the end of the quarter for Net Sales generated by Celgene and its Affiliates, and within [***] of the end of the quarter for Net Sales generated by Sublicensees. Royalty and other payments for each calendar quarter will be due at the same time as such written reports for the calendar quarter. The reports will include, at a minimum, the following information for the applicable calendar quarter, [***].

(c) *Records and Audits*. Celgene will keep, and will cause each of the other Selling Parties, as applicable, to keep, and Bluebird will keep, adequate books and records of accounting for the purpose of calculating all royalties and other amounts payable by either Party to the other Party hereunder and ensuring each Party's compliance hereunder. For the [***] following the end of the calendar year to which each will pertain, such books and records of accounting (including those of the other Selling Parties, as applicable) will be kept at each of their principal place of business. At the request of either Party, the other Party will, and, with respect to Celgene, Celgene will cause each of the other Selling Parties to, permit the requesting Party and its representatives (including an independent auditor), at reasonable times and upon reasonable notice, to examine the books and records maintained pursuant to this Section 4.4(c). Such examinations may not [***]. Except as provided below, the cost of this examination will be borne by [***]. Unless disputed as described below, if such audit concludes that additional payments were owed or that excess payments were made during such period, [***]. In the event of a dispute regarding such books and records, [***] Bluebird and Celgene will work in good faith to resolve the disagreement. If the Parties are

unable to reach a mutually acceptable resolution of any such dispute within [***] such dispute will be resolved in accordance with [***].

(d) *Currency Exchange*. With respect to Net Sales invoiced in U.S. dollars, the Net Sales and the amounts due to Bluebird hereunder will be expressed in U.S. dollars. With respect to Net Sales invoiced in a currency other than U.S. dollars, payments will be calculated based on [***].

(e) [***].

(f) *Blocked Payments*. In the event that, by reason of applicable Law in any country, it becomes impossible or illegal for Celgene (or any other Selling Party) to transfer, or have transferred on its behalf, payments owed Bluebird hereunder, Celgene will [***].

(g) *Interest Due*. If any payment due to either Party under this License Agreement is overdue (and is not subject to a good faith dispute), then such paying Party will pay interest thereon (before and after any judgment) at an annual rate (but with interest accruing on a daily basis) of [***].

(h) *Mutual Convenience of the Parties*. The royalty and other payment obligations set forth hereunder have been agreed to by the Parties for the purpose of reflecting and advancing their mutual convenience, including the ease of calculating and paying royalties and other amounts to Bluebird.

5. Ownership and Inventorship of IP.

5.1 Solely-Owned IP. Subject to Section 5.2, as between the Parties, each Party will own and retain all right, title and interest in and to any and all Know-How and Patents arising therefrom that are discovered, created, conceived, developed or reduced to practice solely by or on behalf of such Party under or in connection with this License Agreement, including as part the Celgene Development & Commercialization Program ("Solely Owned IP"). Subject to the licenses hereunder and the other terms and conditions of this License Agreement, each Party will be solely responsible for the Prosecution and Maintenance, and the enforcement and defense, of any Patents within its Solely Owned IP, and the other Party will have no rights with respect thereto.

5.2 Joint IP. The Parties will jointly own any and all Know-How and Patents arising therefrom that are discovered, created, conceived, developed or reduced to practice jointly by or on behalf of the Parties, under or in connection with this License Agreement, including as part of the Celgene Development & Commercialization Program ("Joint IP"). Each Party will have an undivided one-half interest in and to Joint IP. Each Party will exercise its ownership rights in and to such Joint IP, including the right to license and sublicense or otherwise to exploit, transfer or encumber its ownership interest, without an accounting or obligation to, or consent required from, the other Party, but subject to the licenses hereunder and the other terms and conditions of this License Agreement, including Section 3.4. At the reasonable written request of a Party, the other Party will in writing grant such consents and confirm that no such accounting is required to effect the foregoing regarding Joint IP. Each Party, for itself and on behalf of its Affiliates, licensees and Sublicensees, and employees, subcontractors, consultants and agents of any of the foregoing, hereby assigns (and to the extent such assignment can only be made in the future hereby agrees to assign), to the other Party a joint and undivided interest in and to all Joint IP. The Prosecution and Maintenance, and the enforcement and defense, of any Patents within Joint IP will be jointly managed by the Parties on mutually agreeable terms to be entered into by the Parties at the time any such Patents are first filed, provided that (a) all recoveries and Patent Costs arising from the enforcement or defense of any

Patents within Joint IP, absent further agreement, will be shared by the Parties in accordance with Section 7.2(e) (provided that sufficient advance written notice of any such Patent Costs is given to the Party not incurring same) and (b) Patent Costs incurred in connection with the Prosecution and Maintenance of Patents within Joint IP will be apportioned as set forth in Sections 6.1 and 6.3, provided that in each case ((a) and (b)), if either Party elects not to pay any such Patent Costs for any such Patent, the Parties will meet and agree upon an equitable way to treat such Patent.

5.3 Inventorship. Inventorship determination for all Patents worldwide arising from any Know-How created, conceived or developed by or on behalf of the Parties under or in connection with this License Agreement and thus the ownership thereof will be made in accordance with applicable United States patent Laws.

5.4 Allocation. Notwithstanding Sections 5.1 – 5.3, the Patent Committee may allocate ownership of a particular item of intellectual property to improve the prospects of obtaining patent protection with respect to such item of intellectual property, even if such allocation is not in accordance with the terms of Sections 5.1 – 5.3, so long as the Parties mutually agree to such allocation.

6. Patent Prosecution and Maintenance.

6.1 Generally. Subject to Sections 6.2 and 6.3, Bluebird will have the sole right to Prosecute and Maintain Patents within the Licensed IP. Bluebird will use commercially reasonable efforts to, where applicable and upon Celgene's reasonable request, separate parent Patent applications within the Licensed IP into one or more separate Patent applications for Specific Patents, to the extent permitted under applicable Law, where doing so would not reasonably be expected to materially harm any Patent within the Licensed IP or other Patents owned by Bluebird or its Affiliates, provided that the foregoing limitation will not apply to Licensed IP that is Collaboration IP. Bluebird will be responsible for [***]. Celgene will be responsible for [***]. Except for costs associated with [***] during the License Agreement Term Celgene will be responsible for [***].

6.2 Celgene Input. Bluebird will regularly provide Celgene with copies of all applications for Patents within the Licensed IP, and all other material submissions and correspondence with any patent authorities regarding such Patents, in sufficient time to allow for review and comment by Celgene. In addition, Bluebird will provide Celgene and its counsel with an opportunity to consult with Bluebird and its counsel regarding Prosecution and Maintenance of any such Patents in the Field, and Bluebird will consider in good faith all comments timely made by Celgene and its counsel. In the event of any disagreement between any of Bluebird or Celgene, Bluebird will have the final decision-making authority with respect to the matter involved as long as Bluebird acts in good faith.

6.3 Specific Patents. For any Patent within the Licensed IP [***] (each "Specific Patent"), the following will apply: upon Celgene's written request, and provided that Bluebird reasonably agrees with Celgene that the following Prosecution and Maintenance activities would not materially harm any other Patent within the Licensed IP or other Patents owned by Bluebird or its Affiliates (other than Collaboration IP), Celgene will control the Prosecution and Maintenance of the Specific Patents, and notwithstanding anything in Section 6.1 to the contrary, Celgene will be solely responsible for the payment of all related Patent Costs. In addition, Celgene will provide Bluebird and its counsel with an opportunity to consult with Celgene and its counsel regarding Prosecution and Maintenance of any such Specific Patents, and Celgene will include or reflect all reasonable comments timely made by Bluebird and its counsel. Celgene acknowledges and agrees that Bluebird

may grant similar rights to other exclusive Third Party licensees under any Patent within the Licensed IP that has claims Covering only a product that is not a Licensed Product (or its manufacture or use) and no other product (or its manufacture or use), other than Specific Patents. If the Parties cannot agree whether or not any Patent within the Licensed IP is a Specific Patent, or if Bluebird claims that the foregoing Prosecution and Maintenance activities would materially harm any other Patent within the Licensed IP or other Patents owned by Bluebird or any of its Affiliates, either of the Parties may refer such dispute to a mutually agreeable, disinterested, conflict-of-interest-free individual not affiliated or consulting with either Party and who has at least fifteen (15) years of patent prosecution experience in the pharmaceutical field. Any such arbitration will be conducted under the then-current rules of the American Arbitration Association, and the decision of the arbitrator will be final.

6.4 Election Not to Prosecute or Maintain or Pay Patent Costs. If Bluebird elects not (a) to Prosecute or Maintain any Patents within the Licensed IP in any particular country before the applicable filing deadline or continue such activities once filed in a particular country, or (b) to pay the Patent Costs associated with Prosecution or Maintenance of any Patents within the Licensed IP, then in each such case Bluebird will so notify Celgene, promptly in writing and in good time to enable Bluebird to meet any deadlines by which an action must be taken to preserve such Patent in such country, if Celgene so requests. Upon receipt of each such notice by Bluebird, Celgene will have the right, but not the obligation, to notify Bluebird in writing on a timely basis that Celgene will assume control of the Prosecution or Maintenance of such Patent, and bear the Patent Costs thereafter incurred by Celgene with respect thereto. In addition, Celgene will provide Bluebird and its counsel with an opportunity to consult with Celgene and its counsel regarding Prosecution and Maintenance of any such Patents, and Celgene will include or reflect all reasonable comments timely made by Bluebird and its counsel. If after making such election, Celgene elects not to pay the Patent Costs associated with Prosecution or Maintenance of any such Patent, then in each such case Celgene will so notify Bluebird and on the ninetieth (90^{th}) day after Bluebird's receipt of such notice such Patent will no longer be licensed to Celgene hereunder and will no longer be included within the "Licensed IP" hereunder.

6.5 Third Party Rights. To the extent that a Third Party licensor of Bluebird has retained any right to Prosecute or Maintain any Patent within the Licensed IP licensed to Celgene hereunder (including pursuant to an Applicable Bluebird In-License), or otherwise be involved in such activities, Bluebird will use commercially reasonable efforts to cause such Third Party licensor to take the actions specified by this Section 6 (including Sections 6.6 and 6.7) in a manner consistent with the in-license applicable thereto, but Bluebird will not be deemed to be in breach of its obligations under this Section 6 if, after using such commercially reasonable efforts, it is unable to comply with such obligations because of actions taken or not taken by such Third Party licensor.

6.6 Patent Extensions. Subject to the remainder of this Section 6.6, if any election for patent term restoration or extension, supplemental protection certificate or any of their equivalents may be made with respect to any Patent within the Licensed IP, after consultation with Celgene, the Parties will discuss and seek to reach mutual agreement whether or not to take such action. If the Parties are not able to reach mutual agreement, (a) Celgene will have the sole right to make the final decision whether or not to seek such patent term restoration or extension, supplemental protection certificate or any of their equivalents with respect to Specific Patents and Patents within the Collaboration IP licensed to Celgene hereunder and (b) Bluebird will have the sole right to make the final decision whether or not to seek such patent term restoration or extension, supplemental

protection certificate or any of their equivalents with respect to all other Patents within the Licensed IP.

6.7 Regulatory Exclusivity Periods. With respect to any Patent listings required for any Regulatory Exclusivity Periods for Product, the Parties will mutually agree on which Patents within the Licensed IP to list, provided that if the Parties are not able to agree, Celgene will have the right to make the final decision, and provided further that the exercise of such right by Celgene will not increase or otherwise change the rights or obligations of the Parties hereunder.

6.8 Cooperation. Each Party will reasonably cooperate with the other Party in the Prosecution and Maintenance of Patents within the Licensed IP. Such cooperation includes promptly executing all documents, or requiring inventors, subcontractors, employees and consultants and agents of Celgene and Bluebird and their respective Affiliates and Sublicensees to execute all documents, as reasonable and appropriate so as to enable the Prosecution and Maintenance of any such Patents in any country.

6.9 Patent Marking. Celgene will mark, and will cause all other Selling Parties to mark, Product with all Patents within the Licensed IP in accordance with applicable Law, which marking obligation will continue for as long as (and only for as long as) required under applicable Law.

6.10 Common Interest Disclosures. With regard to any information or opinions disclosed pursuant to this License Agreement by one Party to the other Party regarding Prosecution and Maintenance of Patent within the Licensed IP, or enforcement of intellectual property and/or technology by or against Third Parties, Bluebird and Celgene agree that they have a common legal interest in determining the ownership, scope, validity and/or enforcement of the Licensed IP, and whether, and to what extent, Third Party intellectual property rights may affect the conduct of the Development and Commercialization of any Licensed Product, and have a further common legal interest in defending against any actual or prospective Third Party claims based on allegations of misuse or infringement of intellectual property rights relating to the Development or Commercialization of any Licensed Product. Accordingly, the Parties agree that all such information and materials obtained by the Parties from each other will be used solely for purposes of the Parties' common legal interests with respect to the conduct of the Agreement. All such information and materials will be treated as protected by the attorney-client privilege, the work product privilege, and any other privilege or immunity that may otherwise be applicable. By sharing any such information and materials, neither Party intends to waive or limit any privilege or immunity that may apply to the shared information and materials. Neither Party will have the authority to waive any privilege or immunity on behalf of the other Party without such other Party's prior written consent, nor will the waiver of privilege or immunity resulting from the conduct of one Party be deemed to apply against any other Party. This Section 6.10 will be subject to any right granted by either Party to any Third Party, provided that the grant of such right to such Third Party does not conflict with the other Party's rights or the first Party's obligations under this License Agreement.

7. Patent Enforcement and Defense.

7.1 Notice. Each Party will promptly notify, in writing, the other Party upon learning of any actual or suspected Competitive Infringement of any Patents within the Licensed IP by a Third Party, or of any claim of invalidity, unenforceability, or non-infringement of any Patents within the Licensed IP, and will, along with such notice, supply the other Party with any evidence in its possession pertaining thereto. For purposes of this License Agreement, "Competitive Infringement"

means any allegedly infringing activity in the Field (which, for the purposes of this definition, will include all indications and will not be limited to cancer) with respect to a Patent within the Licensed IP, which activity (a) falls within the scope then in effect of the licenses granted by Bluebird to Celgene as set forth in Sections 3.1, (b) is subject to Section 7.2(f), or (c) would be competitive with a Licensed Product and targets the same Target Antigen as such Licensed Product.

7.2 Enforcement and Defense.

(a) *Patents within the Licensed IP and Competitive Infringement*.

(i) As between the Parties, [***] will have the first right, but not the obligation, to seek to abate any Competitive Infringement of the Patents within the Licensed IP by a Third Party, or to file suit against any such Third Party for such Competitive Infringement. If [***] does not take steps to abate such Competitive Infringement, or file suit to enforce the Patents within the Licensed IP against such Third Party with respect to such Competitive Infringement, within a commercially reasonably time, [***] will have the right (but not the obligation) to take action to enforce the Patents within the Licensed IP against such Third Party for such Competitive Infringement. [***] will pay all its Patent Costs incurred for such enforcement.

(ii) Neither Party will exercise any of its enforcement rights under this Section 7.2(a) without first consulting with the other Party, provided that this consultation requirement will not limit either Party's rights under this Section 7.2(a).

(b) *Defense*. As between the Parties, [***] will have the first right, but not the obligation, to defend against a declaratory judgment action or other action challenging any Patents within the Licensed IP, other than with respect to [***]. If [***] does not take steps to defend within a commercially reasonably time, or elects not to continue any such defense (in which case it will promptly provide notice thereof to [***] then [***] will have the right (but not the obligation) to defend any such Patent.

(c) *Withdrawal, Cooperation and Participation*. With respect to any infringement or defensive action identified above in this Section 7.2:

(i) [***].

(e) *Damages*. Unless otherwise agreed by the Parties, all monies recovered upon the final judgment or settlement of any action described in Section 7.2(a) or any action described in Section 7.2(b) will be used first to [***] with the balance of any such recovery to be divided as follows:

(i) To the extent such recovery reflects [***]

(ii) To the extent such recovery reflects [***]

(iii) For the remainder of any such recovery, [***].

(f) *Biosimilar Applications.* If either Party receives a copy of an application submitted to the FDA under subsection (k) of Section 351 of the Public Health Service Act ("PHSA") (a "Biosimilar Application") naming Licensed Product as a reference product or otherwise becomes aware that such a Biosimilar Application has been filed (such as in an instance described in Section 351(1)(9)(C) of the PHSA), such Party will, [***].

7.3 Third Party Rights. To the extent that a Third Party licensor of Bluebird has retained any right to (a) defend against a declaratory judgment action or other action challenging any Patents

within the Licensed IP, (b) seek to abate any Competitive Infringement of the Patents within the Licensed IP by a Third Party, or (c) take any other actions described in Section 7.2(f) for any Patent within the Licensed IP licensed to Celgene hereunder (including pursuant to an Applicable Bluebird In-License), or otherwise be involved in such activities, Bluebird will use commercially reasonable efforts to cause such Third Party licensor to take the actions specified by this Section 7.3 in a manner consistent with the in-license applicable thereto, but Bluebird will not be deemed to be in breach of its obligations under this Section 7.3 if, after using such commercially reasonable efforts, it is unable to comply with such obligations because of actions taken or not taken by such Third Party licensor.

8. Confidentiality.

The Parties acknowledge and agree that terms of this License Agreement and all Materials, ideas and information of any kind, whether in written, oral, graphical, machine-readable or other form, whether or not marked as confidential or proprietary, which are transferred, disclosed or made available by a Party or at the request of a Party, including any of the foregoing of Third Parties, will be subject to the provisions of Section 8 of the Master Collaboration Agreement, the terms of which survive during the License Agreement Term and for [***] thereafter. Notwithstanding Section 8 of the Master Collaboration Agreement, data arising from Clinical Studies conducted under the License Agreement relating to the Elected Candidate or Licensed Product ("**Clinical Data'**) shall be the Confidential Information of both Parties and each Party may use such Clinical Data for internal purposes including for the research and development of their Independent Target Antigen Program, provided that neither Party may publish or otherwise publicly disclose the Clinical Data without the prior written consent of the other Party. Bluebird will issue a press release promptly following the Amendment Effective Date, in the form attached hereto as Appendix E. A redacted version of this License Agreement will be agreed to by the Parties and shall be consistent with the corresponding redacted version of this License Agreement in such manner as is provided in Section 8.3 of the Master Collaboration Agreement.

9. Warranties; Limitations of Liability; Indemnification.

9.1 Representations and Warranties. Each Party represents and warrants to the other as of the Original License Agreement Effective Date and as of the Amendment Effective Date that it has the legal right and power to enter into this License Agreement, to extend the rights and licenses granted or to be granted to the other in this License Agreement, and to fully perform its obligations hereunder.

9.2 Additional Representations and Warranties of Bluebird. Except as set forth in Schedule 9.2, Bluebird represents and warrants to Celgene that, as of the Original License Agreement Effective Date:

(a) *Licensed IP*. Appendix F sets forth a complete and accurate list of all Patents included in the Licensed IP, indicating the owner, licensor and/or co-owner(s), if applicable, and, for any Elected Candidate and Licensed Product-relevant subject matter or Materials, if no Patent is specifically licensed, a list of all subject matter or Materials that are included in the Licensed IP, including those licensed under a materials use license or equivalent. Bluebird Controls the Patents listed on Appendix F and the Know-How within the Licensed IP, and is entitled to grant the licenses specified herein. Bluebird has not granted to any Third Party any rights or licenses under such Patents or Know-How within the Licensed IP that would conflict with the licenses granted to Celgene hereunder.

(b) *Third Party Agreements*. The Applicable Bluebird In-Licenses are valid and binding obligations of Bluebird and, to the Knowledge of Bluebird, the applicable licensor, enforceable against Bluebird and, to the Knowledge of Bluebird, the applicable licensor, in accordance with their terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally. Neither Bluebird nor any of its Affiliates has received any notice of any counterparty's intention to terminate any Applicable Bluebird In-License in whole or in part or any notice requesting any amendment, alteration or modification of such Applicable Bluebird In-License or any sublicense or assignment thereunder. There is no breach or default, or event which upon notice or the passage of time, or both, could give rise to any breach or default, in the performance of any Applicable Bluebird In-License by Bluebird or any of its Affiliates or, to the Knowledge of Bluebird, the counterparty thereto, and Bluebird has not received any notice of any such breach, default or event. Except for the Applicable Bluebird In-Licenses, neither Bluebird nor any of its Affiliates is a party to any license, sublicense or other agreement pursuant to which Bluebird or such Affiliate has received a license or other rights relating to the Elected Candidate or Licensed Product. All Patents and Know-How licensed to Bluebird under the Applicable Bluebird In-Licenses are Controlled by Bluebird for purposes of the licenses granted to Celgene under this License Agreement.

(c) *Patents*. To Bluebird's Knowledge, the Patents listed on Appendix F have been procured or are being procured from the respective patent offices in accordance with applicable Law. None of the Patents included in the Licensed IP is or has been involved in any opposition, cancellation, interference, reissue or reexamination proceeding, and no Licensed IP is the subject of any judicial, administrative or arbitral order, award, decree, injunction, lawsuit, proceeding or stipulation. Neither Bluebird nor any of its Affiliates has received any notice alleging that the Patents in the Licensed IP are invalid or unenforceable, or challenging Bluebird's ownership of or right to use any such rights.

(d) *No Conflicts*. The execution, delivery and performance by Bluebird of this License Agreement and the consummation of the transactions contemplated hereby will not result in any violation of, conflict with, result in a breach of or constitute a default under any understanding, contract or agreement to which Bluebird is a party or by which it is bound. Neither Bluebird nor any of its Affiliates has entered into any agreement or otherwise licensed, granted, assigned, transferred, conveyed or otherwise encumbered or disposed of any right, title or interest in or to any of its assets, including any intellectual property rights, that would in any way conflict with or impair the scope of any rights or licenses granted to Celgene hereunder.

(e) *Outlicenses.* Appendix G sets forth a complete and accurate list of all agreements relating to the licensing, sublicensing or other granting of rights by Bluebird to any Person with respect to the Licensed IP and the Target Antigen, and Bluebird has provided complete and accurate copies of all such agreements to Celgene. Except for the Applicable Bluebird In-Licenses, Bluebird and its Affiliates are not subject to any payment obligations to Third Parties as a result of the execution or performance of this License Agreement. Neither Bluebird nor any of its Affiliates has granted any liens or security interests on the Licensed IP and the Licensed IP is free and clear of any mortgage, pledge, claim, security interest, covenant, easement, encumbrance, lien or charge of any kind.

(f) *No Proceedings*. There is no action, suit, proceeding or investigation pending or, to the Knowledge of Bluebird, currently threatened in writing against or affecting Bluebird that

questions the validity of this License Agreement or the right of Bluebird to enter into this License Agreement or consummate the transactions contemplated hereby.

(g) *No Infringement*. Neither Bluebird nor any of its Affiliates has received any notice of any claim that any Patent, Know-How or other intellectual property Controlled by a Third Party would be infringed or misappropriated by the production, use, research, Development, Manufacture or Commercialization of the Elected Candidate or Licensed Product pursuant to this License Agreement, and, to the Knowledge of Bluebird, there are no Patents, Know-How or other intellectual property owned by a Third Party and not included in the Licensed IP or In-Licensed IP that are necessary for the production, use, research, Development, Manufacture or Commercialization of Elected Candidate or Licensed Product.

9.3 Disclaimers. Without limiting the respective rights and obligations of the Parties expressly set forth herein, each Party specifically disclaims any guarantee that any Licensed Product will be successful, in whole or in part. EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS LICENSE AGREEMENT, THE PARTIES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, WITH RESPECT TO ANY PATENTS, KNOW-HOW, ELECTED CANDIDATE OR LICENSED PRODUCT, INCLUDING WARRANTIES OF VALIDITY OR ENFORCEABILITY OF ANY PATENT RIGHTS, TITLE, QUALITY, MERCHANTABILITY, FITNESS FOR A PARTICULAR USE OR PURPOSE, PERFORMANCE, AND NONINFRINGEMENT OF ANY THIRD PARTY PATENTS OR OTHER INTELLECTUAL PROPERTY RIGHTS.

9.4 [***].

9.5 Performance by Others. The Parties recognize that each Party may perform some or all of its obligations under this License Agreement through Affiliates and permitted subcontractors provided, however, that each Party will remain responsible and liable for the performance by its Affiliates and permitted subcontractors and will cause its Affiliates and permitted subcontractors to comply with the provisions of this License Agreement in connection therewith.

9.6 Indemnification.

(a) *Indemnification by Celgene*. Celgene will indemnify Bluebird, its Affiliates and their respective directors, officers, employees, Third Party licensors and agents, and their respective successors, heirs and assigns (collectively, "Bluebird Indemnitees"), and defend and save each of them harmless, from and against any and all losses, damages, liabilities, costs and expenses (including reasonable attorneys' fees and expenses) (collectively, "Losses") in connection with any and all suits, investigations, claims or demands of Third Parties (collectively, "Third Party Claims") against the Bluebird Indemnitees arising from or occurring as a result of: (i) the material breach by Celgene of any term of this License Agreement; (ii) any gross negligence or willful misconduct on the part of Celgene in performing its obligations under this License Agreement; or (iii) the Development or Commercialization by or on behalf of Celgene or any of its Affiliates or Sublicensees of Elected Candidate or Licensed Product, except in each case for those Losses for which Bluebird has an obligation to indemnify Celgene pursuant to Section 9.6(b), as to which Losses each Party will indemnify the other to the extent of their respective liability; provided, however, that Celgene will not be obligated to indemnify Bluebird Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of an Bluebird Indemnitee.

(b) *Indemnification by Bluebird*. Bluebird will indemnify Celgene, its Affiliates and their respective directors, officers, employees and agents, and their respective successors, heirs and assigns (collectively, "Celgene Indemnitees"), and defend and save each of them harmless, from and against any and all Losses in connection with any and all Third Party Claims against Celgene Indemnitees arising from or occurring as a result of: (i) the material breach by Bluebird of any term of this License Agreement; (ii) any gross negligence or willful misconduct on the part of Bluebird in performing its obligations under this License Agreement; or (iii) the Development by or on behalf of Bluebird or any of its Affiliates or Sublicensees of Elected Candidate or Licensed Product, except in each case for those Losses for which Celgene has an obligation to indemnify Bluebird pursuant to Section 9.6(a), as to which Losses each Party will indemnify the other to the extent of their respective liability for the Losses; provided, however, that Bluebird will not be obligated to indemnify Celgene Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of a Celgene Indemnitee.

(c) *Notice of Claim*. All indemnification claims provided for in Sections 9.6(a) and 9.6(b) will be made solely by such Party to this License Agreement (the "Indemnified Party"). The Indemnified Party will promptly notify the indemnifying Party (an "Indemnification Claim Notice") of any Losses or the discovery of any fact upon which the Indemnified Party intends to base a request for indemnification under Sections 9.6(a) and 9.6(b), but in no event will the indemnifying Party be liable for any Losses that result from any delay in providing such notice. Each Indemnification Claim Notice must contain a description of the claim and the nature and estimated amount of such Loss (to the extent that the nature and amount of such Loss is known at such time). The Indemnified Party will furnish promptly to the indemnifying Party copies of all papers and official documents received in respect of any Losses and Third Party Claims.

(d) *Defense, Settlement, Cooperation and Expenses*.

(i) *Control of Defense*. At its option, the indemnifying Party may assume the defense of any Third Party Claim by giving written notice to the Indemnified Party within thirty (30) days after the indemnifying Party's receipt of an Indemnification Claim Notice, provided however that (A) the Third Party Claim solely seeks monetary damages and (B) the indemnifying Party expressly agrees in writing that as between the indemnifying Party and the Indemnified Party, the indemnifying Party will be solely obligated to satisfy and discharge the Third Party Claim in full and is able to reasonably demonstrate that it has sufficient financial resources (the matters described in (A) and (B), the "Litigation Conditions"). The assumption of the defense of a Third Party Claim by the indemnifying Party will not be construed as an acknowledgment that the indemnifying Party is liable to indemnify the Indemnified Party in respect of the Third Party Claim, nor will it constitute a waiver by the indemnifying Party of any defenses it may assert against the Indemnified Party's claim for indemnification. Upon assuming the defense of a Third Party Claim, the indemnifying Party may appoint as lead counsel in the defense of the Third Party Claim any legal counsel selected by the indemnifying Party (the indemnifying Party will consult with the Indemnified Party with respect to a possible conflict of interest of such counsel retained by the indemnifying Party). In the event the indemnifying Party assumes the defense of a Third Party Claim, the Indemnified Party will immediately deliver to the indemnifying Party all original notices and documents (including court papers) received by the Indemnified Party in connection with the Third Party Claim. Should the indemnifying Party assume the defense of a Third Party Claim, except as provided in Section 9.6(d)(ii), the indemnifying Party will not be liable to the Indemnified Party for any legal costs or expenses subsequently incurred by such Indemnified Party in connection with the analysis, defense or

settlement of the Third Party Claim. The Indemnified Party may, at any time, assume the defense of a Third Party Claim if at any time the Litigation Conditions are not satisfied with respect to such Claim. In the event that it is ultimately determined that the indemnifying Party is not obligated to indemnify, defend or hold harmless the Indemnified Party from and against the Third Party Claim, the Indemnified Party will reimburse the indemnifying Party for any and all costs and expenses (including attorneys' fees and costs of suit) and any Third Party Claims incurred by the indemnifying Party in its defense of the Third Party Claim.

(ii) *Right to Participate in Defense*. Without limiting Section 9.6(d)(i), any Indemnified Party will be entitled to participate in, but not control, the defense of such Third Party Claim and to employ counsel of its choice for such purpose; provided, however, that such employment will be at the Indemnified Party's own cost and expense unless (A) the employment thereof has been specifically authorized by the indemnifying Party in writing, (B) the indemnifying Party has failed to assume the defense and employ counsel in accordance with Section 9.6(d)(i) (in which case the Indemnified Party will control the defense), (C) the interests of the Indemnified Party and the indemnifying Party with respect to such Third Party Claim are sufficiently adverse to prohibit the representation by the same counsel of both Parties under applicable Law, ethical rules or equitable principles, or (D) the indemnifying Party no longer satisfies the Litigation Conditions, in which case the indemnifying Party will assume [***] of any such costs and expenses of counsel for the Indemnified Party.

(iii) *Settlement*. With respect to any Third Party Claims that relate solely to the payment of money damages in connection with a Third Party Claim and that will not result in the Indemnified Party's becoming subject to injunctive or other relief or otherwise adversely affecting the business of the Indemnified Party in any manner, and as to which the indemnifying Party will have acknowledged in writing the obligation to indemnify the Indemnified Party hereunder, and subject to the Litigation Conditions being satisfied, the indemnifying Party will have the sole right to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss, on such terms as the indemnifying Party, in its sole discretion, will deem appropriate. With respect to all other Losses in connection with Third Party Claims, where the indemnifying Party has assumed the defense of the Third Party Claim in accordance with Section 9.6(d)(i), the indemnifying Party will have authority to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss provided it obtains the prior written consent of the Indemnified Party (such consent not to be unreasonably withheld, delayed or conditioned). The indemnifying Party will not be liable for any settlement or other disposition of a Loss by an Indemnified Party that is reached without the prior written consent of the indemnifying Party. Regardless of whether the indemnifying Party chooses to defend or prosecute any Third Party Claim, no Indemnified Party will admit any liability with respect to or settle, compromise or discharge, any Third Party Claim without the prior written consent of the indemnifying Party, such consent not to be unreasonably withheld, delayed or conditioned.

(iv) *Cooperation*. If the indemnifying Party chooses to defend or prosecute any Third Party Claim, the Indemnified Party will, and will cause each other Indemnified Party to, cooperate in the defense or prosecution thereof and will furnish such records, information and testimony, provide such witnesses and attend such conferences, discovery proceedings, hearings, trials and appeals as may be reasonably requested in connection therewith. Such cooperation will include access during normal business hours afforded to indemnifying Party to, and reasonable retention by the Indemnified Party of, records and information that are reasonably relevant to such Third Party Claim, and making Indemnified Parties and other employees and agents available on a

mutually convenient basis to provide additional information and explanation of any material provided hereunder, and the indemnifying Party will reimburse the Indemnified Party for all its reasonable out-of-pocket costs and expenses in connection therewith.

(v) *Costs and Expenses*. Except as provided above in this Section 9.6(d), the costs and expenses, including attorneys' fees and expenses, incurred by the Indemnified Party in connection with any claim will be reimbursed on a calendar quarter basis by the indemnifying Party, without prejudice to the indemnifying Party's right to contest the Indemnified Party's right to indemnification and subject to refund in the event the indemnifying Party is ultimately held not to be obligated to indemnify the Indemnified Party.

9.7 Insurance. Each Party will maintain at its sole cost and expense, an adequate liability insurance or self-insurance program (including product liability insurance) to protect against potential liabilities and risk arising out of activities to be performed under this License Agreement, and any agreement related hereto and upon such terms (including coverages, deductible limits and self-insured retentions) as are customary in the U.S. pharmaceutical industry for the activities to be conducted by such Party under this License Agreement. Subject to the preceding sentence, such liability insurance or self-insurance program will insure against all types of liability, including personal injury, physical injury or property damage arising out of the manufacture, sale, use, distribution or marketing of Licensed Product. The coverage limits set forth herein will not create any limitation on a Party's liability to the other under this License Agreement.

10. Term and Termination.

10.1 Term. This License Agreement will commence as of the Original License Agreement Effective Date and, unless sooner terminated in accordance with the terms hereof or by mutual written consent, will continue until there are no more payments owed Bluebird on Licensed Product in the United States (the "License Agreement Term"). Upon there being no more such payments hereunder for any such Licensed Product in the United States, the licenses to Celgene contained in Section 3.1 for such Licensed Product both in the United States and ROW will be perpetual and fully paid up (subject to reimbursement to Bluebird of In-License Payments pursuant to Section 4.1) and will remain exclusive with respect to Licensed Product in all such countries.

10.2 Termination by Bluebird.

(a) *Breach*. Bluebird will have the right to terminate this License Agreement in full upon delivery of written notice to Celgene in the event of any material breach by Celgene of any terms and conditions of this License Agreement in a manner that fundamentally frustrates the transactions contemplated by this License Agreement, provided that such termination will not be effective if such breach, has been cured within [***] after written notice thereof is given by Bluebird to Celgene specifying the nature of the alleged breach (or, if such default cannot be cured within such [***] period, within [***] after such notice if Celgene commences actions to cure such default within such [***] period and thereafter diligently continues such actions, but fails to cure the default by the end of such [***]; provided, however, that to the extent such material breach involves the failure to make a payment when due, such breach must be cured within [***] after written notice thereof is given by Bluebird to Celgene.

(b) *Termination for IP Challenge*. Bluebird will have the right to terminate this License Agreement in full upon written notice to Celgene in the event that Celgene or any of its Affiliates or Sublicensees directly or indirectly challenges in a legal or administrative proceeding the

patentability, enforceability or validity of any Patents within the Licensed IP (except as a defense against a claim, action or proceeding asserted by Bluebird against Celgene or its Affiliates or Sublicensees) (a "Patent Challenge"); provided that with respect to any such Patent Challenge by any Sublicensee of Celgene, (i) Bluebird will not have the right to terminate this License Agreement under this Section 10.2(b) if Celgene (A) causes such Patent Challenge to be terminated or dismissed or (B) terminates such Sublicensee's sublicense to the Patents being challenged by the Sublicensee, in each case ((A) and (B)) within [***] of Bluebird's notice to Celgene under this Section 10.2(b), and (ii) Bluebird may terminate this License Agreement only with respect to the country or countries in which such Sublicensee has commenced a Patent Challenge unless such country or countries are the United States, France, Germany, Italy, Spain and/or the United Kingdom, in which case Bluebird may terminate this entire License Agreement. In the event Celgene intends to assert a Patent Challenge in any forum, not less than [***] prior to making any such assertion, Celgene will provide to Bluebird a complete written disclosure of each basis known to Celgene for such assertion. Notwithstanding the foregoing, Bluebird's termination right under this Section 10.2(b) will not apply to any Affiliate of Celgene that first becomes an Affiliate of Celgene after the Effective Date of this License Agreement in connection with a Business Combination, where such Affiliate of Celgene was undertaking activities in connection with a Patent Challenge prior to such Business Combination; provided however that Celgene causes such Patent Challenge to terminate within forty-five (45) days after such Business Combination.

10.3 Termination by Celgene.

(a) *Breach*. Celgene will have the right to terminate this License Agreement in full upon delivery of written notice to Bluebird in the event of any material breach by Bluebird of any terms and conditions of this License Agreement in a manner that fundamentally frustrates the transactions contemplated by this License Agreement, provided that such termination will not be effective if such breach has been cured within [***] after written notice thereof is given by Celgene to Bluebird specifying the nature of the alleged breach (or, if such default cannot be cured within such [***] period, within [***] after such notice if Bluebird commences actions to cure such default within such [***] period and thereafter diligently continues such actions, but fails to cure the default by the end of such [***].

(b) *Discretionary Termination*. Beginning with the [***] Celgene will have the right to terminate this License Agreement in full at its discretion for any reason by delivering written notice to Bluebird, such termination to be effective [***] following the date of such notice.

(c) *Alternative to Termination Under Section 10.3(a)*. If Celgene has the right to terminate this License Agreement under Section 10.3(a) (including expiration of all applicable cure periods thereunder), in lieu of exercising such termination right, Celgene may elect once by written notice to Bluebird before the end of such applicable cure period to have this License Agreement continue in full force and effect and instead have, starting immediately after the end of such applicable cure period, any future Milestone Payments set forth in Section 4.2 and the royalty rates set forth in the table set forth in Section 4.3(a) be reduced by [***], provided that such reduction will not apply if such future Milestone Payments and royalty rates have already been reduced pursuant to Section 11.4(c) of the Master Collaboration Agreement.

10.4 Effects of Termination. Upon termination (but not expiration pursuant to Section 10.1) of this License Agreement for any reason:

(a) *Wind Down*. Celgene will responsibly wind-down, in accordance with accepted pharmaceutical industry norms and ethical practices, any on-going clinical studies for which it has responsibility hereunder in which patient dosing has commenced or, if reasonably practicable and requested by Bluebird, allow Celgene, its Affiliates or its Sublicensees to complete such trials. Celgene will be responsible for any costs associated with such wind-down. Bluebird will pay all costs incurred by either Party to complete such studies should Bluebird request that such studies be completed.

(b) *Sublicenses*. A termination of this License Agreement will not automatically terminate any sublicense granted by Celgene pursuant to Section 3.3 for Commercialization rights with respect to a non-Affiliated Sublicensee, provided that (i) such Sublicensee is not then (A) in material breach of any provision of this License Agreement or (B) in material breach of the applicable sublicense agreement or otherwise in breach of such sublicense agreement in a manner that would give rise to a right of termination on the part of Celgene, (ii) if Bluebird terminates this License Agreement pursuant to Section 10.2(a) for Celgene's failure to fulfill its payment obligations hereunder, such Sublicensee agrees to and does pay to Bluebird all outstanding amounts that accrued as a result of such Sublicensee's activities under the sublicense, (iii) Bluebird will have the right to step into the role of Celgene as sublicensor under any such sublicense executed after the Original License Agreement Effective Date, with all the rights that Celgene had under such sublicense, solely with respect to the Licensed IP, prior to termination of this License Agreement (including the right to receive any payments to Celgene by such Sublicensee that accrue from and after the date of the termination of this License Agreement solely with respect to the Licensed IP), (iv) such Sublicensee will pay to Bluebird all amounts that Celgene would have been obligated to pay to Bluebird hereunder with respect to such Sublicensee's activities had this License Agreement not terminated (less any amounts received by Bluebird in clause (iii) above) and (v) the survival of such sublicense will not result in an imposition of any additional obligations on the part of Bluebird that are not included within the scope of this License Agreement. Celgene will include in any sublicense agreement executed after the Original License Agreement Effective Date that relates solely to the Licensed IP a provision in which said Sublicensee acknowledges its obligations to Bluebird under this Section 10.4(b).

(c) *Cessation of Rights*. Except as otherwise expressly provided in Section 10.4(b), all rights and licenses granted by Bluebird to Celgene in Section 3 will terminate, and Celgene and its Affiliates and Sublicensees will cease all use of Licensed IP and all Development, Manufacture and Commercialization of Elected Candidate and Licensed Product.

(d) *Regulatory Approvals*. To the extent permitted by applicable Law, and subject to Bluebird paying commercially reasonable compensation to Celgene for the assets to be transferred pursuant to this Section 10.4(d) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 10.4(g) below, and such compensation to be reduced by [***] from what would be commercially reasonable compensation if this License Agreement is terminated by Bluebird pursuant to Section 10.2(a)), all Regulatory Approvals and other regulatory filings and communications owned (in whole or in part) or otherwise Controlled by Celgene and its Affiliates and Sublicensees solely relating to the Elected Candidate and/or Licensed Product, and all other documents solely relating to and necessary to further Develop and Commercialize Elected Candidate and Licensed Product, as such items exist as of the effective date of such termination (including all solely related completed and ongoing clinical studies) will be assigned to Bluebird, and Celgene will provide to Bluebird one (1) copy of the foregoing and all documents contained in or

referenced in any such items, together with the raw and summarized data for any clinical studies (and where reasonably available, electronic copies thereof). In the event of failure to obtain assignment, subject to the Parties agreeing on commercially reasonable compensation for the right to access and reference, Celgene hereby consents and grants to Bluebird the right to access and reference (without any further action required on the part of Celgene, whose authorization to file this consent with any Regulatory Authority is hereby granted) any such item.

(e) *Licenses*. Subject to Bluebird paying (i) commercially reasonable compensation to Celgene for the licenses to be granted pursuant to subsection (A) of this Section 10.4(e) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 10.4(g) below, and such compensation to be reduced by [***] from what would be commercially reasonable compensation if this License Agreement is terminated by Bluebird pursuant to Section 10.2(a)), and (ii) amounts payable to Celgene's applicable licensors as set forth below, Celgene will grant to Bluebird and its Affiliates (A) a worldwide, perpetual and irrevocable, nontransferable (except in connection with a permitted assignment of this License Agreement in accordance with Section 11.12), exclusive license, with the right to grant sublicenses through multiple tiers (subject to Section 3.3(b), *mutatis mutandis*), under the Celgene Licensed Product IP, and (B) an exclusive sublicense under the Celgene Licensed Product In-Licensed IP, in each case ((A) and (B)) to the extent such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP are used in or Cover the Licensed Product as of the effective date of termination and to the extent such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP exist as of the effective date of such termination (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP) solely to the extent necessary to research, Develop, Manufacture and Commercialize the Elected Candidate and Licensed Product. With respect to grants of a sublicense under subsection (B) above, Bluebird will be responsible for all amounts payable to the applicable licensor, excluding maintenance fee payments, payments that are trigged by the grant of a sublicense (but including payments triggered by further grants of sublicenses by Bluebird or its sublicensees) and Patent Costs, that are attributable to Bluebird as a sublicensee thereunder under this License Agreement and Celgene will pay same and Bluebird will reimburse Celgene for [***] of such payments within thirty (30) days of receipt of Celgene's written invoice therefor. Celgene will provide Bluebird with copies of all applicable Celgene Licensed Product In-Licenses promptly following the effective date of the termination of this License Agreement. The Prosecution and Maintenance and enforcement and defense rights and obligations of the Parties with respect to any Patents licensed or sublicensed to Bluebird pursuant to this Section 10.4(e) will be discussed and agreed to by the Parties, with the understanding that such Prosecution and Maintenance and enforcement and defense rights and obligations will be substantially similar to those set forth in Section 6, with the roles of Bluebird and Celgene reversed (and such other changes as are appropriate from the context, and taking into account any rights retained by a Third Party licensor of Celgene to Prosecute and Maintain or enforce and defend any Patent sublicensed to Bluebird under this Section 10.4(e)). Bluebird will abide, and will cause all its Affiliates and applicable sublicensees to abide, by all requirements of each Celgene Licensed Product In-License under which Bluebird is sublicensed under this Section 10.4(e) in all material respects (and in any case in all respects in the case that failure to so abide would result in a breach under the Celgene Licensed Product In-License), to the extent applicable to sublicensees thereunder and to the extent disclosed by Celgene to Bluebird, with the understanding that disclosure by Celgene of any Celgene Licensed Product In-License to Bluebird

will be deemed disclosure of such requirements of such Celgene Licensed Product In-License to Bluebird.

(f) *Trademarks*. Subject to Bluebird paying commercially reasonable compensation to Celgene for the license to be granted pursuant to this Section 10.4(f) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 10.4(g) below, and such compensation to be reduced by [***] from what would be commercially reasonable compensation if this License Agreement is terminated by Bluebird pursuant to Section 10.2(a)), Celgene will exclusively license to Bluebird any registered or unregistered trademarks or internet domain names that are specific to and solely used for the Licensed Product worldwide (it being understood that the foregoing will not include any trademarks or internet domain names that contain the corporate or business name(s) of Celgene).

(g) *Commercially Reasonable Compensation*. If the Parties are unable to agree on the amount of commercially reasonable compensation payable by Bluebird to Celgene pursuant to Sections 10.4(d), 10.4(e) or 10.4(f) within ten (10) days of the effective date of termination of this License Agreement, [***].

(h) *Country Termination*. If this License Agreement is terminated only with respect to a specific country pursuant to Section 10.2(b), the provisions of this Section 10.4 will apply only with respect to such terminated country.

10.5 Survival. In addition to the termination consequences set forth in Section 10.4, the following provisions will survive termination or expiration of this License Agreement: Sections 1, 3.3 (mutatis mutandis with respect to licenses granted to Bluebird under Section 10.4), 3.6, 3.7, 4.4, 5, 8, 9.3, 9.4, 9.6, 9.7, 10.1 (last sentence), 10.4, 10.5 and 11. Termination or expiration of this License Agreement will not relieve the Parties of any liability or obligation which accrued hereunder prior to the effective date of such termination or expiration nor preclude either Party from pursuing all rights and remedies it may have hereunder or at law or in equity with respect to any breach of this License Agreement nor prejudice either Party's right to obtain performance of any obligation. All other rights and obligations will terminate upon expiration of this License Agreement.

10.6 Right to Set-off. Notwithstanding anything to the contrary in this License Agreement, each Party has the right at all times to retain and set off against all amounts due and owing to the other Party as determined in a final judgment any damages recovered by such Party for any Losses incurred by such Party.

11. General Provisions.

11.1 Cumulative Remedies and Irreparable Harm. All rights and remedies of the Parties hereunder will be cumulative and in addition to all other rights and remedies provided hereunder or available by agreement, at law or otherwise. Each Party acknowledges and agrees that breach of any of the terms or conditions of this License Agreement would cause irreparable harm and damage to the other and that such damage may not be ascertainable in money damages and that as a result thereof the non-breaching Party would be entitled to seek from a court equitable or injunctive relief restraining any breach or future violation of the terms contained herein by the breaching Party without the necessity of proving actual damages or posting bond. Such right to equitable relief is in addition to whatever remedies either Party may be entitled to as a matter of law or equity, including money damages.

11.2 Business Combination and IP.

(a) *Bluebird Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this License Agreement, no Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Bluebird or any of its Affiliates prior to a Business Combination of Bluebird will be Controlled for purposes of this License Agreement after such Business Combination of Bluebird, other than (i) Applicable Bluebird In-Licenses to the extent in effect immediately prior to such Business Combination of Bluebird, (ii) Collaboration IP, and (iii) any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Bluebird will be Controlled thereafter no matter when such Patent is filed or issued.

(b) *Celgene Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this License Agreement, no Know-How, Materials, Patents Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Celgene or any of its Affiliates prior to a Business Combination of Celgene will be Controlled for purposes of this License Agreement after such Business Combination of Celgene, other than Collaboration IP, and except that any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Celgene will be Controlled thereafter no matter when such Patent is filed or issued.

11.3 Relationship of Parties. Nothing in this License Agreement is intended or will be deemed to constitute a partnership, agency, employer-employee or joint venture relationship between the Parties. No Party will incur any debts or make any commitments for the other, except to the extent, if at all, specifically provided therein. There are no express or implied third party beneficiaries hereunder (except for Bluebird Indemnitees and Celgene Indemnitees for purposes of Section 9.6).

11.4 Compliance with Law. Each Party will perform or cause to be performed any and all of its obligations or the exercise of any and all of its rights hereunder in good scientific manner and in compliance with all applicable Law. Without limiting the foregoing, Bluebird will comply with comply with all applicable Laws and regulations (including U.S. Foreign Corrupt Practices Act and any other applicable anti-bribery or anti-kickback laws or regulations).

11.5 Force Majeure. Neither Party will be liable to the other for failure of or delay in performing obligations set forth in this License Agreement (other than any obligation to pay monies when due), and neither will be deemed in breach of such obligations, if such failure or delay is due to natural disasters or any causes reasonably beyond the control of such Party; provided that the Party affected will promptly notify the other of the force majeure condition and will exert reasonable efforts to eliminate, cure or overcome any such causes and to resume performance of its obligations as soon as possible.

11.6 Governing Law. This License Agreement will be governed by and construed in accordance with the Laws of the State of New York, without respect to its conflict of laws rules, provided that any dispute relating to the scope, validity, enforceability or infringement of any Patents or Know-How will be governed by, and construed and enforced in accordance with, the substantive Laws of the jurisdiction in which such Patents or Know-How apply.

11.7 Counterparts; Facsimiles. This License Agreement may be executed in one or more counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. Facsimile or PDF execution and delivery of this License Agreement

by either Party will constitute a legal, valid and binding execution and delivery of this License Agreement by such Party

11.8 Headings. All headings in this License Agreement are for convenience only and will not affect the meaning of any provision hereof.

11.9 Waiver of Rule of Construction. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this License Agreement. Accordingly, the rule of construction that any ambiguity in this License Agreement will be construed against the drafting party will not apply.

11.10 Interpretation. Whenever any provision of this License Agreement uses the term "including" (or "includes"), such term will be deemed to mean "including without limitation" (or "includes without limitations"). "Herein," "hereby," "hereunder," "hereof" and other equivalent words refer to this License Agreement as an entirety and not solely to the particular portion of this License Agreement in which any such word is used. All definitions set forth herein will be deemed applicable whether the words defined are used herein in the singular or the plural. Unless otherwise provided, all references to Sections and Appendices in this License Agreement are to Sections and Appendices of this License Agreement. References to any Sections include Sections and subsections that are part of the related Section (e.g., a section numbered "Section 2.1" would be part of "Section 2", and references to "Section 2.1" would also refer to material contained in the subsection described as "Section 2.1(a)").

11.11 Binding Effect. This License Agreement will inure to the benefit of and be binding upon the Parties, their Affiliates, and their respective lawful successors and assigns.

11.12 Assignment. This License Agreement may not be assigned by either Party, nor may either Party delegate its obligations or otherwise transfer licenses or other rights created by this License Agreement, except as expressly permitted hereunder or otherwise without the prior written consent of the other Party, which consent will not be unreasonably withheld, delayed or conditioned; provided that without consent (a) Celgene may assign this License Agreement to (i) an Affiliate or (ii) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets, and (a) Bluebird may assign this License Agreement to (i) an Affiliate or (ii) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets or that portion of its business pertaining to the subject matter of this License Agreement; provided further that, except in the case where a Party is involved in a merger or consolidation where it is the surviving entity and no assets of such Party that are subject to this License Agreement have been transferred as a result of such merger or consolidation, (A) such assigning Party provides the other Party to this License Agreement with at least thirty (30) business days advance written notice of such assignment(s) and the assigning Party agrees in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to remain fully liable for the performance of its obligations under this License Agreement by its assignee(s), (B) the assignee(s) agree in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to assume performance of all such assigned obligations, (C) in the case of any assignment by Bluebird, all Licensed IP licensed to Celgene under this License Agreement will be transferred to such assignee(s) effective as of such assignment(s), (D) all of the matters referred to in clauses (A), (B) and (C), as applicable, will be set forth in documentation reasonably acceptable to the non-assigning Party prior to any such assignment(s) (and with such reasonable acceptance not to be unreasonably withheld, conditioned or

delayed) and in all cases will provide the non-assigning Party with the full benefits of its rights under this License Agreement (after taking into account all risks involving applicable counter-party performance and bankruptcy and insolvency risks, including those involving contractual rejection under 11 USC §365) as if no such assignment(s) had occurred, and (E) in the case of any assignment, the assigning Party will reimburse the non-assigning Party for all of the legal fees and expenses incurred by such non-assigning Party in connection with the matters set forth in clause (D) of this sentence in an aggregate amount not to exceed [***] and provided, further, that if Bluebird wishes to assign any Licensed IP to its Affiliates, it will be permitted to do so conditioned on each such Affiliate becoming a party to this License Agreement, in the form of an amendment to this License Agreement executed by Celgene, Bluebird and such Affiliate, pursuant to which such Affiliate would agree to assume all obligations hereunder, and grant to Celgene all rights hereunder, with respect to the Licensed IP. The terms of this License Agreement will be binding upon and will inure to the benefit of the successors, heirs, administrators and permitted assigns of the Parties. Any purported assignment in violation of this Section 11.12 will be null and void ab initio.

11.13 Notices. All notices, requests, demands and other communications required or permitted to be given pursuant to this License Agreement will be in writing and will be deemed to have been duly given upon the date of receipt if delivered by hand, recognized international overnight courier, confirmed facsimile transmission, or registered or certified mail, return receipt requested, postage prepaid to the applicable address or facsimile number set forth in Section 13.14 of the Master Collaboration Agreement. Either Party may change its designated address and facsimile number by notice to the other Party in the manner provided in this Section 11.13.

11.14 Amendment and Waiver. This License Agreement may be amended, supplemented, or otherwise modified only by means of a written instrument signed by both Parties; provided that any unilateral undertaking or waiver made by one Party in favor of the other will be enforceable if undertaken in a writing signed by the Party to be charged with the undertaking or waiver. Any waiver of any rights or failure to act in a specific instance will relate only to such instance and will not be construed as an agreement to waive any rights or fail to act in any other instance, whether or not similar.

11.15 Severability. In the event that any provision of this License Agreement will, for any reason, be held to be invalid or unenforceable in any respect, such invalidity or unenforceability will not affect any other provision hereof, and the Parties will negotiate in good faith to modify this License Agreement to preserve (to the extent possible) their original intent.

11.16 Entire Agreement. This License Agreement, together with the Master Collaboration Agreement, is the sole agreement with respect to the subject matter and supersedes all other agreements and understandings between the Parties with respect to same (including Confidential Agreement). In the event of any conflict between the terms of this License Agreement and the terms of the Master Collaboration Agreement, the terms of this License Agreement will control.

11.17 Force Majeure. Neither Celgene nor Bluebird will be liable for failure of or delay in performing obligations set forth in this License Agreement (other than any obligation to pay monies when due), and neither will be deemed in breach of such obligations, if such failure or delay is due to natural disasters or any causes reasonably beyond the control of Celgene or Bluebird and without the fault or negligence of the Party so failing or delaying; provided that the Party affected will promptly notify the other of the force majeure condition and will exert reasonable efforts to eliminate, cure or overcome any such causes and to resume performance of its obligations as soon as possible.

11.18 Celgene Parties. The Parties hereby acknowledge and agree that (a) Celgene Corp is the party to this License Agreement with respect to all rights and obligations under this License Agreement in the United States, provided that with respect to payment obligations under this License Agreement, Celgene Corp is the responsible party with respect to all such payment obligations; (b) Celgene Europe is the party to this License Agreement with respect to all rights and obligations under this License Agreement outside of the United States, provided that with respect to payment obligations under this License Agreement, Celgene Europe is not a responsible party with respect to any such payment obligations; and (c) as between Bluebird, on the one hand, and Celgene Corp and Celgene Europe, on the other, Celgene Corp shall undertake all actions permitted or required to be taken by Celgene Corp and/or Celgene Europe.

11.19 Co-Promotion/Co-Development Option Exercise. To the extent that Bluebird exercises its option to co-promote and co-develop a Licensed Product that is an Optioned Candidate (as defined in the Master Collaboration Agreement) in accordance with, and subject to, Section 5.3 of the Master Collaboration Agreement, Bluebird and Celgene will enter into a Co-Development, Co-Promote and Profit Share Agreement in the form that will be agreed upon by the Parties within twenty (20) days of the Amendment Effective Date (instead of the form attached as Exhibit B of the Master Collaboration Agreement).

[*Remainder of this Page Intentionally Left Blank*]

IN WITNESS WHEREOF, the Parties have caused this License Agreement to be executed by their respective duly authorized officers as of the Amendment Effective Date.

BLUEBIRD BIO, INC.

By: /s/ Jason Cole
(Signature)

Name: Jason Cole

Title: Chief Operating and Legal Officer

Date:

CELGENE CORPORATION

By: /s/ Elizabeth Mily
(Signature)

Name: Elizabeth Mily

Executive Vice President
Title: Strategy and Business Development

Date:

CELGENE EUROPEAN INVESTMENT COMPANY LLC (CEICO)

By: /s/ Elizabeth Mily
(Signature)

Name: Elizabeth Mily
Executive Vice President
Title: Strategy and Business Development

Date:

Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

[***]

Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

Exhibit 10.20

Execution Version

FIRST AMENDMENT TO

AMENDED AND RESTATED CO-DEVELOPMENT, CO-PROMOTE AND PROFIT SHARE AGREEMENT

By and Between

BLUEBIRD BIO, INC.

and

CELGENE CORPORATION

and

CELGENE EUROPEAN INVESTMENT COMPANY LLC

Dated as of May 8, 2020

Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

FIRST AMENDMENT TO CCPS AGREEMENT

This First Amendment to Amended and Restated Co-Development, Co-Promote and Profit Share Agreement (this "**First Amendment**") is entered into as of May 8, 2020 (the "**First Amendment Effective Date**") by and between **bluebird bio, Inc.**, a Delaware corporation having its principal place of business at 60 Binney Street, Cambridge, MA 02142 ("**Bluebird**") and **Celgene Corporation, Inc.**, a corporation organized under the laws of Delaware and having a principal place of business at 86 Morris Avenue, Summit, NJ 07901 ("**Celgene Corp**"), with respect to all rights and obligations under the CCPS Agreement (as defined below) in the United States (subject to Section 18.18 of the **CCPS** Agreement), and **Celgene European Investment Company LLC**, a limited liability company organized under the laws of Delaware and having a principal place of business at Route de Perreux 1, 2017 Boudry, Switzerland, with respect to all rights and obligations under the CCPS Agreement outside of the United States (subject to Section 18.18 of the CCPS Agreement) ("**Celgene Europe**" and together with Celgene Corp, "**Celgene**"). Celgene and Bluebird are sometimes referred to herein individually as a "**Party**" and collectively as the "**Parties**". Capitalized terms not defined herein shall have the meaning provided in the CCPS Agreement, and if not defined in the CCPS Agreement, in the Master Collaboration Agreement.

BACKGROUND

WHEREAS, the Parties have entered into an Amended and Restated Co-Development, Co-Promote and Profit Share Agreement dated March 26, 2018 (the "**CCPS Agreement**");

WHEREAS, the Parties wish to amend the CCPS Agreement with respect to the Manufacture and Supply of Vectors, payments and royalties outside of the U.S., and exclusivity in accordance with the terms and conditions set forth below.

NOW THEREFORE, in consideration of the foregoing premises and the mutual promises, covenants and conditions contained in this First Amendment, the Parties agree as follows:

ARTICLE 1

Definitions

1.1 New Definitions. The following definitions are hereby added to Article 1 of the CCPS Agreement:

1.58 "**Adherent Vector**" means Vector manufactured utilizing the [***] systems process for incorporation into Elected Candidate and Licensed Products for Development and Commercialization thereof.

1.59 "**Suspension Transition Plan**" has the meaning set forth in Section 7.4(c)(i).

1.60 "**Suspension Vector**" means Vector manufactured utilizing [***] for incorporation into Elected Candidate and Licensed Products for Development and Commercialization thereof.

1.61 "**Suspension Vector Supplies**" means supplies of Suspension Vectors and associated Payloads Manufactured for incorporation into Elected Candidate and Licensed Products for Development or Commercialization thereof.

1.62 "**Vector**" means recombinant lentiviral agent(s) (including all components therein other than Payloads) for gene therapy intended to deliver a nucleotide sequence, including those recombinant viral agent(s) (including all components therein other than Payloads) for any Elected Candidate or

Licensed Product. For avoidance of doubt, Vectors do not include Payloads. "Vectors" refer to both Adherent Vectors and Suspension Vectors."

1.2 Amendment to the Definitions Table. The definitions table following Section 1.57 of the CCPS Agreement is hereby amended by adding the following additional definitions to the table:

Defined Terms	***Location in the CCPS Agreement (as amended by the First Amendment)***
Adherent Vector	Section 1.58
Aldevron	Section 7.4(c)(viii)
Brammer	Section 7.4(b)(i)
Brammer Agreement	Section 7.4(b)(i)
Clinical Data	Section 15
Eurogentec	Section 7.4(c)(viii)
First Amendment Effective Date	First Amendment, Introduction
Independent Target Antigen Program	Section 10.4(a)
Manufacturing and Supply Agreement	Section 7.4(c)(ii)
Suspension Transition Plan	Section 1.59
Suspension Vector	Section 1.60
Suspension Vector Supplies	Section 1.61
Transaction Agreements	First Amendment, Section 2.2
Transition Period	Section 7.4(b)(ii)
Transition Plan	Section 7.4(b)(ii)
Vector	Section 1.62

1.3 Amendment to the Definitions Table. The definitions table following Section 1.57 is hereby amended by deleting the following definitions from the table:

Defined Terms	***Location***
Biosimilar Product	Section 1.4
Business Acquisition	Section 10.4
Business Party	Section 10.4
Business Program	Section 10.4
Milestone Event	Section 11.2(a)
Milestone Payment	Section 11.2(a)
Worldwide Commercialization Plan	Section 1.56

1.4 Amendment of Existing Definitions. Article 1 of the CCPS Agreement is hereby amended as follows:

(a) The definition of "**Biosimilar Product**" is hereby amended by deleting the existing text and replacing it with the following text:

"1.4 [Reserved]."

(b) The definition of "**Licensed Product**" is hereby amended by deleting the existing text and replacing it with the following text:

"1.34 "**Licensed Product**" means any product that constitutes or incorporates an Elected Candidate (including all modified and improved versions thereof), in all forms, presentations, and formulations (including manner of delivery and dosage)."

(c) The definition of "**Worldwide Commercialization Plan**" is hereby amended by deleting the existing text and replacing it with the following text:

"1.56 [Reserved]."

1.5 Joint Governance; Limits on JGC Authority. Section 3.1(e) of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"(e) *Limits on JGC Authority*. Each Party will retain the rights, powers and discretion granted to it under this CCPS Agreement and no such rights, powers, or discretion will be delegated to or vested in the JGC unless such delegation or vesting of rights is expressly provided for in this CCPS Agreement or the Parties expressly so agree in writing. The JGC will not have the power to, nor will the Party having the tie-breaking vote in the JGC have the power to (i) amend, modify or waive compliance with this CCPS Agreement (other than as expressly permitted hereunder), (ii) alter, increase or expand the Parties' rights or obligations under this CCPS Agreement (other than as permitted by Section 2.2), (iii) determine that a Party has fulfilled any obligations under this CCPS Agreement or that a Party has breached any obligation under this CCPS Agreement, or (iv) make a decision that is expressly stated to require the mutual agreement of the Parties. For avoidance of doubt, the JGC will have no right to supervise or direct the Development and Commercialization of Elected Candidate or Licensed Product for ROW Administration, and Celgene will have sole decision-making authority with respect to such Development and Commercialization, including with respect to the ROW Development & Commercialization Program."

1.6 This first paragraph of Section 4.2 of the CCPS Agreement is hereby amended by deleting t the existing text and replacing it with the following text:

"4.2 Development Plan. The Parties acknowledge that as of the Effective Date, Celgene has prepared and delivered to Bluebird an initial U.S. Development Plan, and the JGC will review and approve such initial U.S. Development Plan, with the goal of coordinating and harmonizing the U.S. Development Plan with the ROW Development Plan. Thereafter, Celgene will update the U.S. Development Plan each calendar year [***] and the JGC will review and approve any such update or any other amendment to the U.S. Development Plan. In addition, either Party may request at any time that the JGC consider and approve other updates to the U.S. Development Plan. Promptly after the CCPS Agreement Effective Date, Celgene will prepare an initial ROW Development Plan and will provide it to the JGC for purposes of discussion and the goal of coordinating and harmonizing the U.S. Development Plan and the ROW Development Plan. From the First Amendment Effective Date, Celgene will update the ROW Development Plan each calendar year submit it to the JGC [***]. Notwithstanding anything in this CCPS Agreement to the contrary, the Parties acknowledge and agree that (i) Bluebird may decline to perform any Development activity proposed to be conducted by Bluebird (excluding Manufacturing of Suspension Vectors and associated Payloads to the extent that Bluebird is responsible for such Manufacture pursuant to this CCPS Agreement (as amended) or any agreement entered into by the Parties in relation to such Manufacture), and (ii) the U.S. Development Plan will not include, and Bluebird will have no obligation to perform, any Development activity that Bluebird has declined to perform (other than the Manufacture of Suspension Vectors and associated Payloads to the extent that Bluebird is responsible for such Manufacture pursuant to this CCPS Agreement (as amended) or any agreement entered into by the Parties in relation to such Manufacture), provided that once Bluebird has agreed to perform a Development activity, it will be obligated to perform, and cannot decline to perform, such activity. Further:

(a) The JGC will set the required form and contents of the U.S. Development Plan.

(b) Neither Party (itself or by or through any others, including any Affiliates or Sublicensees) will take any material action regarding the Development of Elected Candidate or Licensed Product for U.S. Administration unless described in the U.S. Development Plan, provided that the foregoing will not restrict Celgene from taking any action regarding the Development of Elected Candidate or Licensed Product for ROW Administration.

(c) All Development of Elected Candidate and Licensed Product for U.S. Administration will be conducted under the supervision of the JGC and as part of the U.S. Development & Commercialization Program.

(d) All Development of Elected Candidate and Licensed Product for ROW Administration will be conducted under the sole control of Celgene and as part of the ROW Development & Commercialization Program. At each calendar quarter meeting of the JGC, Celgene will provide the JGC with an update on the material events regarding the Development of Elected Candidate and Licensed Product by Celgene for ROW Administration.

(e) Celgene will prepare and maintain, and will cause its Affiliates and Sublicensees to prepare and maintain, reasonably complete and accurate records regarding the Development of Elected Candidate and Licensed Product for ROW Administration. Annually, Celgene will provide the JGC with a reasonably-detailed report regarding the Development of Elected Candidate and Licensed Product for ROW Administration. Such report will contain sufficient detail to enable Bluebird to assess Celgene's compliance with its Development and Commercialization obligations hereunder or as may be applicable to enable Bluebird to comply with the Applicable Bluebird In-Licenses. In addition to the foregoing, Celgene will provide Bluebird with such additional information regarding any such activities as Bluebird

may reasonably request from time to time to the extent reasonably necessary to enable Bluebird to comply with Applicable Bluebird In-Licenses. Bluebird shall transmit to Celgene samples of historical reports issued to the licensors under the Applicable Bluebird In-Licenses.

1.7 All references to Worldwide Commercialization Plan in the CCPS Agreement will, from the First Amendment Effective Date be read as references to the U.S. Commercialization Plan.

1.8 The phrase "(other than Manufacturing of Vectors and associated Payloads)" in Section 5.2 of the CCPS Agreement is hereby amended and replaced by the following text:

"(other than Manufacturing of Vectors and associated Payloads to the extent that Bluebird is responsible for such Manufacture pursuant to this CCPS Agreement (including the transition plan attached hereto) or any agreement entered into by the Parties in relation to such Manufacture)"

1.9 Section 5.4 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"5.4 Solely to the extent necessary to enable Bluebird to comply with the Applicable Bluebird In-Licenses, Celgene, directly or through one or more of its Affiliates or Sublicensees, will use Commercially Reasonable Efforts, (i) to Develop Licensed Product in the Field for ROW Administration and to obtain Regulatory Approvals therefor; and (ii) to Commercialize Licensed Product in the Field for ROW Administration after obtaining such Regulatory Approval, in each country in the ROW where Regulatory Approval has been obtained."

1.10 The Parties agree that Section 5.6 only applies to the promotion of Elected Candidate and Licensed Product for U.S. Administration.

1.11 Section 7.1 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"7.1 Generally.

(a) As of and after the CCPS Agreement Effective Date, subject to the terms and conditions of this CCPS Agreement, (i) the Parties will assume through the JGC joint responsibility for (1) Manufacture of Elected Candidate and Licensed Product for Development and (2) Manufacture of Licensed Product for Commercialization for U.S. Administration, each under the Development & U.S. Commercialization Program, and (ii) Celgene will assume sole responsibility for Manufacturing Licensed Product for Commercialization for ROW Administration, and (iii) subject to Section 7.4, Celgene will purchase Suspension Vector Supply from Bluebird or its authorized designee for such purposes (pursuant to Section 7.4(c)). The Joint Manufacturing Committee (JMC), established by the JGC in accordance with Section 3.1(c)(iv) of the CCPS Agreement, shall be maintained during the CCPS Agreement Term. Notwithstanding the foregoing, subject to, and with effect from, the expiry or termination of the Manufacturing and Supply Agreement, Celgene will assume sole responsibility for the Manufacture of Licensed Product for Commercialization for U.S. Administration and ROW Administration (including Vectors and associated Payloads for U.S. Administration and ROW Administration) in accordance with this CCPS Agreement.

(b) Subject to the terms and conditions of this CCPS Agreement (and including without limitation the Transition Plan), as of and after the First Amendment Effective Date, Celgene will assume sole responsibility for Manufacturing Adherent Vector for Development and Commercialization of Elected Candidate and Licensed Product in the Field for U.S. Administration (with respect to such U.S.

Administration under the supervision of the JGC in accordance with Article 3) and ROW Administration."

1.12 Section 7.4 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"7.4 Vector Manufacturing. Notwithstanding anything else in this Section 7:

(a) *Generally*. As of the First Amendment Effective Date but subject to the other clauses of this Section 7.4, (and with respect to U.S. Administration under the supervision of the JGC in accordance with Article 3) Celgene will be solely responsible for the Manufacture and supply of Adherent Vector and associated Payload for the Development and Commercialization of Elected Candidate and Licensed Products in the Field for U.S. Administration and ROW Administration, and Manufacture and supply of Suspension Vector and associated Payload for Development and Commercialization of Elected Candidate and Licensed Products in the Field for ROW Administration, subject to the other clauses of this Section 7.4 and subject to the respective obligations of Bluebird and Celgene under the Manufacturing and Supply Agreement and any other agreements entered into by the Parties in relation to Payloads. Subject to Section 7.4(c), Bluebird will be primarily responsible for Manufacture of Suspension Vector Supply for the Development and Commercialization of Elected Candidate and Licensed Product for U.S. Administration and will collaborate in good faith with Celgene and use Commercially Reasonable Efforts to Manufacture Suspension Vector as a secondary source for the Development and Commercialization for ROW Administration as required under the Manufacturing and Supply Agreement. Solely in connection with such "back-up" or "second source" rights under the Manufacturing and Supply Agreement, Celgene (or its designee) will be Celgene's secondary source of Suspension Vector and associated Payload for Development and Commercialization of Elected Candidate and Licensed Product in the Field for U.S. Administration and primary source of Suspension Vector and associated Payload for the Development and Commercialization of Elected Candidate and Licensed Product in the Field for ROW Administration following completion of the Suspension Transition Plan. Notwithstanding anything herein to the contrary, subject to, and with effect from, the expiry or termination of the Manufacturing and Supply Agreement, Celgene will assume sole responsibility for the Manufacture and supply of Suspension Vector including associated Payloads for the Development and Commercialization of Elected Candidate and Licensed Product for U.S. Administration and ROW Administration in accordance with this CCPS Agreement."

(b) *Adherent Vector Technology Transfer*.

(i) On and with effect on the First Amendment Effective Date, Bluebird shall assign to Celgene or its designated Affiliate the [***] pursuant to the terms and conditions of an assignment agreement or notice agreed in advance with Celgene.

(ii) Each Party shall use Commercially Reasonable Efforts to perform activities ascribed to it in the transition plan set forth in Appendix L, (the "**Transition Plan**") to transfer to Celgene Adherent Vector Manufacturing (including associated Payloads) responsibilities. Following successful completion of the Transition Plan, Celgene shall be the sole point of contact with Brammer regarding the day-to-day operations relating to the Adherent Vector, including all interactions with Regulatory Authorities relating to Adherent Vector. All costs incurred by the Parties in relation to the execution of the Transition Plan will be apportioned in accordance with Schedule 4.3(b). The Parties will mutually agree on the forecast for Adherent Vector to be Manufactured for U.S. Administration.

(iii) Within [***] of Celgene's written request or such other timeframe agreed by the Parties in writing, Bluebird shall initiate transfer of QC assays for Adherent Vector to a

Celgene or a Third Party selected by Celgene, provided such Third Party is under written obligations of confidentiality and non-use at least as stringent as those contained herein.

(iv) Bluebird and Celgene shall each be responsible for [***] provided that the Parties shall share equally in any recovered fees related thereto. Cost of Adherent Vector Manufacture and associated Payloads shall be included in the Cost of Goods Sold (for clarity, as a component of the Manufacturing Costs).

(c) *Suspension Vector Supply Terms*.

(i) Bluebird shall use Commercially Reasonable Efforts to qualify its manufacturing facility for the Manufacture of Suspension Vector for U.S. Administration and ROW Administration. Unless otherwise agreed by the Parties in writing, within [***] the Parties will negotiate in good faith a transfer plan to be agreed by the Parties, to engage in a technology transfer as set forth in Section 7.4(c)(v) (the "**Suspension Transition Plan**"). The Parties will use Commercially Reasonable Efforts to finalize the Suspension Transition Plan within [***]. The Parties shall commence the technology transfer activities referred to in such Suspension Transition Plan within [***]. From the date of [***] and subject to the terms and conditions of the Manufacturing and Supply Agreement, Bluebird shall solely be responsible for the Manufacture of Suspension Vector and associated Payloads for U.S. Administration and ROW Administration. After completion of the Suspension Transition Plan, Bluebird and its Affiliates will be primarily responsible for the Manufacture of Suspension Vector and associated Payloads for all Elected Candidate and Licensed Product required for clinical Development and Commercialization in the Field for U.S. Administration, and Bluebird will collaborate in good faith and use Commercially Reasonable Efforts to be Celgene's secondary source of supply for the Manufacture of Suspension Vector and associated Payloads for Elected Candidate and Licensed Product required for clinical Development and Commercialization in the Field for ROW Administration in each case, solely in connection with such "back-up" or "business continuity source" rights under the Manufacturing and Supply Agreement.

(ii) The Parties will enter into a Manufacturing and Supply Agreement, between each other or among the Parties and an Affiliate, covering Suspension Vector Supply within [***] of the First Amendment Effective Date, which agreement will be consistent with the terms of this Section 7.4(c) and will otherwise be subject in all respects to the terms and conditions of this CCPS Agreement (the "**Manufacturing and Supply Agreement**").

(iii) The cost to Celgene of Suspension Vector Supply for Commercialization for ROW Administration will equal [***] of Bluebird's Fully Burdened Manufacturing Cost for such Manufacture, plus [***] unless otherwise agreed by the Parties in writing. The Manufacturing Cost of Suspension Vector Supply for Commercialization for U.S. Administration will be included in the Cost of Goods Sold (for clarity, as a component of the Manufacturing Costs). The cost of Suspension Vector Supply for Development will be included in the U.S. Development Costs, subject to adjustment as provided therein.

(iv) The Manufacturing and Supply Agreement will include the terms set forth in Appendix J, including license grants from Celgene to Bluebird under the Celgene Licensed IP to the extent necessary or useful for Bluebird to Manufacture Suspension Vector Supply.

(v) In accordance with Section 7.4(c)(i), and as set forth in Appendix J, Bluebird will use Commercially Reasonable Efforts to engage in a technology transfer to allow Celgene to Manufacture Suspension Vector (through the first commercial batch of Suspension Vector) itself or

by through its designated Third Party manufacturer (each, a "**Manufacturing Party**"), by transferring all Know-How and Materials Controlled by Bluebird or its Affiliates that are necessary to Manufacture Suspension Vector. Costs and expenses of the Parties associated with such technology transfer will be [***]. Notwithstanding the foregoing, Bluebird shall only be required to deliver Know-How and Materials in its or its Affiliates' actual possession or under its control and shall not be required to produce or create any additional Know-How or Materials. Before any such transfer, the Manufacturing Party shall enter into a reasonable confidentiality agreement with Bluebird with respect to the use and handling of such Know-How and Materials.

(vi) Celgene will use Commercially Reasonable Efforts to establish a second source of Suspension Vector within [***] of the commencement of the activities under the Suspension Transition Plan, in accordance with the regulatory filing strategy aligned at the JGC.

(vii) Any purchase of Suspension Vector Supply from Bluebird or its designee will expressly not include any license rights to any Know-How or Patents, but instead all licenses (implied, by exhaustion or otherwise) will arise under Section 10.1, if and as applicable.

(viii) For the purpose of this CCPS Agreement, certain words and phrases (and their correlatives) relating to Manufacturing will have the meanings set forth on Appendix J.

(ix) Celgene agrees to collaborate in good faith with Bluebird and use Commercially Reasonable Efforts to Manufacture Suspension Vector for U.S. Administration to the extent circumstances would require Bluebird to activate "business continuity source" supply for U.S. Administration. Bluebird agrees to collaborate in good faith with Celgene and use Commercially Reasonable Efforts to Manufacture Suspension Vector for ROW Administration to the extent circumstances would require Bluebird to activate "business continuity source" supply for ROW Administration pursuant to the Manufacturing and Supply Agreement.

(x) For as long as Bluebird is sole source of supply of Suspension Vector, in the event of any supply deficiency or shortage of Suspension Vector or associated Payload, any available Suspension Vector or Payload supplies shall be allocated for U.S Administration and ROW Administration on pro rata basis, using the forecasted demand for the year in which such deficiency or shortage occurs, unless otherwise agreed by the Parties in writing.

(d) *Payloads.*

(i) Celgene shall have the right to conduct quality audits of Bluebird's existing inventories of Bluebird's of [***] and shall have the right to purchase from Bluebird, at cost, [***] working [***] with sufficient shelf life and in sufficient quantities to allow Celgene to Manufacture Vector in accordance with this CCPS Agreement while Celgene establishes the supply arrangements referred to in Section 7.4(d)(ii).

(ii) Bluebird will take such actions as are necessary to permit Celgene to purchase quantities of plasmids from [***] solely for use in Manufacturing Vector for Elected Candidate and Licensed Products as permitted under this CCPS Agreement, under and pursuant to a supply or similar agreement between Celgene and [***] and [***] respectively, and Bluebird will execute and deliver a letter of authorization or similar document to Aldevron and Eurogentec, respectively, to authorize such purchases. Forecasting for plasmids will be reviewed and approved by the JGC on a quarterly basis. Information received from [***] or [***] relating to the plasmids sequence shall be deemed to be Bluebird's Confidential Information for purposes of this CCPS Agreement. In addition, Bluebird will take such actions as are necessary to permit Celgene to purchase quantities of [***] cells for

use in Manufacturing Vector for Elected Candidate and Licensed Products as permitted under this CCPS Agreement, under and pursuant to a supply or similar agreement between Celgene and [***] and, to the extent required to enable such purchases, Bluebird will execute and deliver a letter of authorization or similar document to [***].

1.13 Section 8.5 of the CCPS Agreement is amended by deleting the reference to "any regulatory milestones".

1.14 Sections 10.1(b), 10.1(c) and the last paragraph of Section 10.1 of the CCPS Agreement are hereby amended by deleting the existing text and replacing it with the following text:

"(b) a worldwide, exclusive (even as to Bluebird, but with respect to Manufacturing, even as to Bluebird only after completion of the technology transfer set forth in Section 7.4(c)(v)) fully paid up, royalty-free license, with the right to sublicense only as permitted by Section 10.3, under Bluebird Licensed IP and Bluebird Regulatory Rights, (i) Develop (including for clarity, Manufacture) Elected Candidate and Licensed Product in the Field for ROW Administration and (ii) to Commercialize (including for clarity Manufacture) Licensed Product in the Field for ROW Administration; and

(c) a worldwide, exclusive royalty-free license, with the right to sublicense only as permitted by Section 10.3, under Bluebird Licensed IP and Bluebird Regulatory Rights, to Manufacture Adherent Vectors and associated Payloads for Licensed Product in the Field for U.S. Administration and ROW Administration.

Further, (i) the foregoing licenses to Bluebird Regulatory Rights include the right to reference same, (ii) the licenses to Commercialize granted in this Section 10.1 will cover only the sale and offer for sale of Licensed Product in finished form and not the sale or offer for sale of Vectors and associated Payloads (other than as and to the extent incorporated in the Licensed Product), and (iii) rights to Manufacture Vectors and associated Payloads are included within the scope of the licenses granted to Celgene under this Section 10.1, which rights are subject to the terms and conditions of Section 7.4."

1.15 Section 10.2 of the CCPS Agreement is hereby amended by deleting the term "Vector" and replacing it with the term "Suspension Vector" throughout.

1.16 Section 10.4 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"10.4 Exclusivity.

(a) Each Party and its Affiliates may research, Develop, Manufacture or Commercialize any actual or potential products (other than Elected Candidate, Licensed Product or, in the case of Celgene and its Affiliates, bb21217) to be used in the Field (which, for the purposes of this Section 10.4(a), will include all indications and will not be limited to cancer) that specifically target the Target Antigen internally or with Third Party collaborators, licensors, licensees or partners (any such program, an "**Independent Target Antigen Program**"), provided that (i) none of the Bluebird Licensed IP or Celgene Licensed IP, as the case may be, or other Patents, Materials or Know-How Controlled by a Party and licensed to the other Party hereunder will be used by such other Party in the conduct of its Independent Target Antigen Programs, (ii) subject to Section 15, none of the Confidential Information of a Party will be used by the other Party in its conduct of Independent Target Antigen Programs, and (iii) each Party conducting an Independent Target Antigen Program will have appropriate internal procedures in place to ensure compliance with provisos (i) and (ii) above."

1.17 Section 10.5 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"10.5 Contract Manufacturers. Subject to the terms and conditions of this CCPS Agreement, either Party will have the right to appoint by a written agreement "contract manufacturers", meaning any Third Party or Affiliate of such Party that Manufactures Licensed Product (or components therefor, including Vector and associated Payloads) for re-sale, but who itself is not a "Sublicensee" hereunder and thereby exercises "have made" rights granted by the other Party hereunder. Subject to the terms and conditions of this CCPS Agreement, either Party will have the right to appoint by a written agreement, "contract research organizations" and other providers performing services on a Party's behalf, none of which will be deemed a "Sublicensee" hereunder. Such Party will be responsible for any such contract manufacturer, contract research organization or service provider hereunder, and further will require any such contract manufacturer, contract research organization or service provider to agree in writing to comply with Sections 10.6 and 15. Each Party can shall have the right to audit and qualify any Third Party contract manufacturer engaged by the other Party. Notwithstanding the foregoing, if, at any time, Bluebird determines that it is appropriate or desirable to outsource the Manufacture of the Suspension Vector for U.S. Administration to a Third Party, and provided that [***] Bluebird shall notify Celgene in writing and shall, before engaging into any request for proposal or similar procurement process, [***]. In the event that Bluebird, after such consultation, determines to engage an alternative or additional manufacturer for the Manufacture of the Suspension Vector for U.S. Administration, Celgene and its Affiliates shall have the right (but not the obligation) to [***].

1.18 Section 11.1 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"11.1 Payments for In-Licenses.

(a) *United States*. With respect to the Development and Commercialization of Elected Candidate and Licensed Product for U.S. Administration hereunder, if any payments become due under any Applicable Pre-Existing In-License, Applicable New In-Licenses, Co-Co In-Licenses or Celgene Licensed Product In-License during the CCPS Agreement Term, the contracting Party thereto will pay same and such payment will be treated as U.S. Development Expenses or Allowable Expenses, as appropriate, provided (i) such payment is not triggered by the grant of a sublicense by the contracting Party under such license agreement to the non-contracting Party under such license agreement (which payment will be borne solely by the contracting Party), (ii) any payment based on any payments made by one Party to the other Party (e.g., sublicense revenue sharing) will be borne solely by the contracting Party, (iii) any payments based on a Business Combination of Bluebird or Celgene will be borne solely by the Party undergoing the Business Combination, (iv) any payments resulting from the contracting Party's breach under such license that is not attributable to the non-contracting Party or any of its contract Third Parties under Section 8.4, or any of its Sublicensees will be excluded, and (v) subject to Section 13.1.

(b) *ROW*. With respect to the Development and Commercialization of Elected Candidate and Licensed Product for ROW Administration hereunder (including the Manufacture of Vectors and associated Payloads therefor pursuant to Section 7.4):

(i) *Applicable Pre-Existing In-Licenses*. If any In-License Payment becomes due under any Applicable Pre-Existing In-License during the CCPS Agreement Term, Bluebird will pay same, provided that Celgene will reimburse Bluebird for any such In-License Payment applicable to ROW Administration within thirty (30) days of Celgene's receipt of Bluebird's written invoice therefor; which In-License Payments (other than payments that are royalties) will not exceed [***] and subject to

Section 13.1. Any such reimbursement by Celgene to Bluebird is in addition to and not in lieu of the other payments required by this Section 11.

(ii) *Applicable New In-Licenses*. Celgene may elect to take a sublicense under any New In-License of Bluebird or its Affiliates and upon such election, such New In-License will be an Applicable New In-License hereunder for all purposes. For the purposes of determining the Parties' respective payment obligations, all Applicable New In-Licenses as of and following the CCPS Agreement Effective Date will be listed on Appendix B. If any In-License Payment becomes due under any Applicable New In-License during the CCPS Agreement Term with respect to ROW Administration, Bluebird will pay same and, subject to Section 13.1, Celgene will reimburse Bluebird for such payment within thirty (30) days of receipt of Bluebird's written invoice therefor. If Celgene elects to convert an Other In-License to an Applicable New In-License pursuant to Section 10.7(b), Celgene will reimburse Bluebird for [***] of any In-License Payments that became due under such Applicable New In-License during the CCPS Agreement Term with respect to ROW Administration to the same extent as if such Applicable New In-License was designated as such as of the CCPS Agreement Effective Date, including with respect to applicable Patent Costs in accordance with Section 6.1, provided that Bluebird provides Celgene with a reasonable accounting of same. To the extent that any grant of a sublicense by Celgene or any Sublicensees under an Applicable New In-License triggers a payment obligation under such Applicable New In-License, Bluebird will pay same and Celgene will reimburse Bluebird for [***] of such payment within thirty (30) days of receipt of Bluebird's written invoice therefor. To the extent that any grant of a sublicense by Bluebird or any Sublicensees under a Celgene Licensed Product In-License triggers a payment obligation under such Celgene Licensed Product In-License, Celgene will pay same and Bluebird will reimburse Celgene for [***] of such payment within thirty (30) days of receipt of Celgene's written invoice therefor.

(iii) If any payments become due under any Co-Co In-Licenses during the CCPS Agreement Term with respect to Licensed Product for ROW Administration, the contracting Party will pay same, and further if Bluebird is the contracting Party, Celgene will reimburse Bluebird for such payment within [***] upon receipt of Bluebird's written invoice therefor, subject to Section 13.1. Any such reimbursement by Celgene to Bluebird is in addition to and not in lieu of the other payments required by this Section 11. "

1.19 Section 11.2 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"11.2 Reserved]."

1.20 Section 11.3 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"11.3 [Reserved]."

1.21 The first sentence of Section 11.5 of the CCPS Agreement is hereby amended by deleting existing text and replacing it with the following text:

"This Section 11.5 will apply solely as it relates to Celgene's payment obligations under Section 11.1, and the reporting obligations related thereto and solely as needed for Bluebird to comply with its obligations under the Bluebird Applicable In-Licenses."

1.22 Section 11.6 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"11.6 [Reserved]."

1.23 Section 14.2(e)(ii)(A) of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"(A) To the extent such recovery reflects lost profits damages, if Celgene was the controlling Party or the Parties jointly controlled, Celgene will retain such lost profits recovery, and if Bluebird was the controlling Party, [***] to Bluebird and [***] to Celgene;"

1.24 Section 15 of the CCPS Agreement is hereby amended by deleting the existing text and replacing it with the following text:

"The Parties acknowledge and agree that terms of this CCPS Agreement and all Materials, ideas and information of any kind, whether in written, oral, graphical, machine-readable or other form, whether or not marked as confidential or proprietary, which are transferred, disclosed or made available by a Party or at the request of a Party, including any of the foregoing of Third Parties, will be subject to the provisions of Section 8 of the Master Collaboration Agreement, the terms of which survive during the CCPS Agreement Term and for ten (10) years thereafter. Notwithstanding Section 8 of the Master Collaboration Agreement, data arising from Clinical Studies conducted under the CCPS Agreement relating to the Elected Candidate or Licensed Product ("**Clinical Data**") shall be the Confidential Information of [***]. A redacted version of this CCPS Agreement will be agreed to by the Parties and shall be consistent with the corresponding redacted version of this CCPS Agreement in such manner as is provided in Section 8.3 of the Master Collaboration Agreement."

1.25 Section 17.1 is hereby amended by deleting the existing text and replacing it with the following text:

"17.1 Term. This CCPS Agreement will commence as of the CCPS Agreement Effective Date and, unless sooner terminated in accordance with the terms hereof or by mutual written consent, will continue until Licensed Product is no longer being Developed or Commercialized in the United States. For all countries (other than the United States), the licenses to Celgene contained in Section 10.1 are perpetual and fully paid up (subject to reimbursement to Bluebird of any In-License Payments pursuant to Section 11.1) and will remain exclusive with respect to Licensed Product in all such countries."

1.26 Section 17.2(c) of the CCPS Agreement is hereby amended by deleting existing text and replacing it with the following text:

(c) *Termination of the Profit & Loss Share*. Bluebird will have the right to terminate the Profit & Loss Share by delivering written notice to Celgene, such termination to be effective [***] following the date of such notice. Promptly following such notice, the Parties will enter into a license agreement with respect to the United States and the ROW, which agreement will be substantially identical to the License Agreement, with such changes that the Parties may, acting reasonably, mutually agree are required in order to address any specific facts or circumstances existing at the time of such termination, provided that such license agreement shall in no event require Celgene to pay any milestone payment or royalties in relation to the Development and Commercialization of Elected Candidate and Licensed Product for ROW Administration. The Parties will enter into such license agreement no later than the effective date of such termination and, if such license agreement is not entered into prior the expiration of such [***] period, upon execution, the effective date of such license agreement will be deemed to be the effective date of such termination. For clarity, (i) termination of the Profit & Loss Share pursuant to this Section 17.2(c) will not release Bluebird from any obligation or liability which, at the time of the effective date of such termination, has already accrued to Celgene or which is attributable to a period prior to the CCPS effective date of such termination, and (ii) any events that have already occurred

before the effective date of such termination (such as achievement of any milestones) will not trigger any payment obligation by Celgene to Bluebird under such executed license agreement.

1.27 Section 17.3(d) of the CCPS Agreement is hereby deleted in its entirety.

1.28 Appendix J is hereby amended by deleting the existing Appendix J and replacing it with the attached Appendix J.

1.29 New Appendices. The following appendices are hereby added to the CCPS Agreement:

Appendix L, attached hereto as Appendix L.

Appendix M, attached hereto as Appendix M.

ARTICLE 2

Payment

2.1 Upfront Payment. As a consideration for this First Amendment and as a consideration for the Parties concurrently with this First Amendment entering into a Second Amended and Restated License Agreement in relation to the product known as bb21217, Celgene Europe shall make a one-time, non-refundable, non-creditable cash payment of two hundred million dollars ($200,000,000) to Bluebird within [***] of the First Amendment Effective Date.

2.2 Taxes. Section 11.5(e) of the CCPS Agreement shall apply to the payment referred to in Section 2.1 of this First Amendment.

ARTICLE 3

Miscellaneous

3.1 [***].

3.2 Each Party represents and warrants to the other as of the date hereof that:

(a) Corporate Power. It is duly organized, validly existing and in good standing under the laws of its jurisdiction of incorporation or formation, and has full corporate power and authority to enter into this First Amendment and to carry out the provisions hereof.

(b) Due Authorization. It is duly authorized to execute and deliver this First Amendment and to perform its obligations hereunder, and the person executing this First Amendment on its behalf has been duly authorized to do so by all requisite corporate action.

(c) Binding Agreement. This First Amendment is legally binding upon it and enforceable against it in accordance with its terms. The execution, delivery and performance of this Amendment by it does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it may be bound, nor violate any applicable Laws.

3.3 Bluebird will issue a press release in the form attached hereto as Appendix N promptly following the First Amendment Effective Date. A redacted version of this First Amendment will be agreed to by the Parties in such manner as is provided in Section 8.3 of the Master Collaboration Agreement.

3.4 Except as otherwise expressly set forth herein, the Agreement shall continue, in full force and effect, in accordance with its terms.

[Signature Page Follows]

IN WITNESS WHEREOF, the Parties have executed this Agreement in duplicate originals by their duly authorized officers as of the First Amendment Effective Date.

bluebird bio, Inc.

By: /s/ Jason Cole

Name: Jason Cole

Title: Chief Operating and Legal Officer

Celgene Corporation

By: /s/ Elizabeth Mily

Name: Elizabeth Mily

Title: Executive Vice President
Strategy and Business Development

Celgene European Investment Company LLC

By: /s/ Elizabeth Mily

Name: Elizabeth Mily

Title: Executive Vice President
Strategy and Business Development

Certain information indicated with [***] in this document has been omitted from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

[***]

Exhibit 31.1

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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 5, 2020

By: /s/ Nick Leschly

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

Exhibit 31.2

CERTIFICATIONS

I, Chip Baird, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 5, 2020

By: /s/ Chip Baird

Chip Baird
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of bluebird bio, Inc. (the "Company") for the period ended June 30, 2020 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: August 5, 2020

By: /s/ Nick Leschly

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

Date: August 5, 2020

By: /s/ Chip Baird

Chip Baird
Chief Financial Officer (Principal Financial Officer and Duly Authorized Officer)