UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2016

OR

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

For the transition period from _____ to _____

Commission File Number 001-37687

EDITAS MEDICINE, INC.
(Exact name of registrant as specified in its charter)

Delaware **(State or other jurisdiction of incorporation or organization)**	**46-4097528** **(I.R.S. Employer Identification No.)**
11 Hurley Street **Cambridge, Massachusetts** **(Address of principal executive offices)**	**02141** **(Zip Code)**

(617) 401-9000
(Registrant's telephone number, including area code)

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

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

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

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

The number of shares of the Common Stock outstanding as of November 1, 2016 was 36,655,936.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements.

Editas Medicine, Inc.
Condensed Consolidated Balance Sheets
(unaudited)
(amounts in thousands, except share and per share data)

	September 30, 2016	December 31, 2015
ASSETS		
Current assets:		
Cash and cash equivalents	$ 199,874	$ 143,180
Accounts receivable	1,122	1,019
Prepaid expenses and other current assets	2,453	786
Total current assets	203,449	144,985
Property and equipment, net	37,072	2,130
Restricted cash and other non-current assets	1,632	2,248
Total assets	$ 242,153	$ 149,363
LIABILITIES, REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities:		
Accounts payable	$ 3,718	$ 1,381
Accrued expenses	9,492	5,456
Deferred rent, current portion	—	88
Other current liabilities	547	—
Total current liabilities	13,757	6,925
Deferred rent, net of current portion	353	—
Deferred revenue	25,800	25,321
Warrant to purchase redeemable securities	—	289
Construction financing lease obligation, net of current portion	32,048	—
Other non-current liabilities	19	27
Total liabilities	71,977	32,562
Commitments and contingencies (see note 6)		
Series A-1 redeemable convertible preferred stock, $0.0001 par value per share: no shares and 21,320,000 shares authorized at September 30, 2016 and December 31, 2015, respectively; no shares and 21,260,000 shares issued and outstanding at September 30, 2016 and December 31, 2015, respectively	—	21,137
Series A-2 redeemable convertible preferred stock, $0.0001 par value per share: no shares and 16,890,699 shares authorized, issued and outstanding at September 30, 2016 and December 31, 2015, respectively	—	59,027
Series B redeemable convertible preferred stock, $0.0001 par value per share: no shares and 26,666,660 shares authorized, issued and outstanding at September 30, 2016 and December 31, 2015, respectively	—	119,751
Stockholders' equity (deficit)		
Preferred stock, $0.0001 par value, 5,000,000 shares and no shares authorized, at September 30, 2016 and December 31, 2015, respectively; no shares issued or outstanding at September 30, 2016 and December 31, 2015, respectively	—	—
Common stock, $0.0001 par value per share: 195,000,000 shares and 92,000,000 shares authorized at September 30, 2016 and December 31, 2015, respectively; 36,651,129 and 4,869,829 shares issued and 35,608,637 and 3,233,638 shares outstanding at September 30, 2016 and December 31, 2015, respectively	4	—
Additional paid-in capital	316,322	5,234
Accumulated deficit	(146,150)	(88,348)
Total stockholders' equity (deficit)	170,176	(83,114)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 242,153	$ 149,363

The accompanying notes are an integral part of the condensed consolidated financial statements.

Editas Medicine, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited)
(amounts in thousands, except per share and share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Collaboration and other research and development revenues	$ 962	$ 670	$ 5,155	$ 837
Operating expenses:				
Research and development	10,832	3,850	30,144	13,020
General and administrative	11,295	4,202	33,215	10,756
Total operating expenses	22,127	8,052	63,359	23,776
Operating loss	(21,165)	(7,382)	(58,204)	(22,939)
Other income (expense), net				
Other income (expense), net	3	21	(22)	(37,219)
Interest income (expense), net	142	(44)	419	(109)
Total other income (expense), net	145	(23)	397	(37,328)
Net loss and comprehensive loss	$ (21,020)	$ (7,405)	$ (57,807)	$ (60,267)
Reconciliation of net loss to net loss attributable to common stockholders:				
Net loss	$ (21,020)	$ (7,405)	$ (57,807)	$ (60,267)
Accretion of redeemable convertible preferred stock to redemption value	—	(104)	(47)	(295)
Net loss attributable to common stockholders	$ (21,020)	$ (7,509)	$ (57,854)	$ (60,562)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.59)	$ (2.57)	$ (1.86)	$ (25.53)
Weighted-average common shares outstanding, basic and diluted	35,505,429	2,925,843	31,040,670	2,371,976

The accompanying notes are an integral part of the condensed consolidated financial statements.

Editas Medicine, Inc.
Condensed Consolidated Statements of Cash Flows
(unaudited)
(amounts in thousands)

	Nine Months Ended September 30,	
	2016	2015
Cash flow from operating activities		
Net loss	$ (57,807)	$ (60,267)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	13,099	1,562
Depreciation	714	323
Non-cash interest expense	—	45
Changes in fair value of warrant liability	87	92
Change in fair value of preferred stock tranche asset or liability	—	35,551
Changes in fair value of anti-dilutive protection liability	—	1,609
Changes in deferred rent	265	(71)
Changes in operating assets and liabilities:		
Accounts receivable	(103)	(1,040)
Prepaid expenses and other current assets	(1,667)	(147)
Other non-current assets	2,235	—
Accounts payable	2,095	(1,080)
Accrued expenses	4,034	823
Deferred revenue	479	25,165
Net cash (used in) provided by operating activities	(36,569)	2,565
Cash flow from investing activities		
Purchases of property and equipment	(2,817)	(1,030)
Changes in restricted cash	(1,619)	—
Net cash used in investing activities	(4,436)	(1,030)
Cash flow from financing activities		
Proceeds from equipment loan, net of issuance costs	—	1,500
Proceeds from the issuance of redeemable convertible preferred stock and tranche rights, net of issuance costs	—	141,711
Payments of equipment loan principal	—	(70)
Proceeds from the issuance of common stock and restricted stock	—	2
Proceeds from initial public offering of common stock, net of issuance costs	97,488	—
Proceeds from stock option exercises	211	—
Net cash provided by financing activities	97,699	143,143
Net increase in cash and cash equivalents	56,694	144,678
Cash and cash equivalents, beginning of period	143,180	10,623
Cash and cash equivalents, end of period	$ 199,874	$ 155,301
Supplemental disclosure of cash and non-cash activities:		
Conversion of preferred stock to common stock upon closing of the initial public offering	$ 199,915	$ —
Capitalization of construction-in-progress related to facility lease obligation	32,595	—
Fixed asset additions included in accounts payable and accrued expenses	244	—
Initial public offering costs incurred but unpaid at period end	—	661
Reclassification of warrants to additional paid-in capital	376	—
Accretion of redeemable convertible preferred stock to redemption value	47	295
Reclassification of liability for common stock subject to repurchase	8	14
Conversion of anti-dilutive protection liability to common stock	—	1,936
Reclassification of preferred stock tranche liability upon settlement	—	37,038
Accrual of final payment fee on equipment loan and debt discount	—	60

The accompanying notes are an integral part of the condensed consolidated financial statements.

Editas Medicine, Inc.
Notes to Condensed Consolidated Financial Statements
(unaudited)

1. Nature of business

Editas Medicine, Inc. (the "Company") is a research stage company dedicated to treating patients with genetically defined diseases by correcting their disease-causing genes. The Company was incorporated in the state of Delaware in September 2013. Its principal offices are in Cambridge, Massachusetts.

Since its inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, and raising capital, and has financed its operations through various equity and debt financings, including the initial public offering of its common stock (the "IPO"), private placements of preferred stock and an equipment loan, and from upfront, milestone and research and development fees paid under a research collaboration with Juno Therapeutics, Inc. ("Juno Therapeutics").

The Company is subject to risks common to companies in the biotechnology industry, including but not limited to, risks of failure of preclinical studies and clinical trials, the need to obtain marketing approval for any drug product candidate that it may identify and develop, the need to successfully commercialize and gain market acceptance of its product candidates, dependence on key personnel, protection of proprietary technology, compliance with government regulations, development by competitors of technological innovations and ability to transition from pilot-scale manufacturing to large-scale production of products.

In February 2016, the Company completed its IPO whereby the Company sold 6,785,000 shares of its common stock, inclusive of 885,000 shares of common stock sold by the Company pursuant to the full exercise of an overallotment option granted to the underwriters in connection with the offering, at a price to the public of $16.00 per share. The shares began trading on the NASDAQ Global Select Market on February 3, 2016. The aggregate net proceeds received by the Company from the offering were $97.5 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company. In connection with the IPO, the board of directors and the stockholders of the Company approved a one-for-2.6 reverse stock split of the Company's issued and outstanding common stock. The reverse stock split became effective on January 15, 2016. All share and per share amounts in the condensed consolidated financial statements have been retroactively adjusted for all periods presented to give effect to the reverse stock split, including reclassifying an amount equal to the reduction in par value to additional paid-in capital. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 24,929,709 shares of common stock. As of September 30, 2016, there were 35,608,637 shares of common stock outstanding. The significant increase in shares outstanding in the first quarter of 2016 is expected to impact the year-over-year comparability of the Company's net loss per share calculations for the next six months.

The Company has incurred annual net operating losses in every year since its inception. The Company had an accumulated deficit of $146.2 million at September 30, 2016, and will require substantial additional capital to fund its operations. The Company has not generated any product revenues and has financed its operations primarily through a public offering, private placements of its equity securities, an equipment loan, and funding from its collaboration with Juno Therapeutics. There can be no assurance that the Company will be able to obtain additional debt or equity financing or generate product revenue or revenues from collaborative partners, on terms acceptable to the Company, on a timely basis or at all. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company's business, results of operations, and financial condition.

2. Summary of significant accounting policies

Unaudited interim financial information

The condensed consolidated financial statements of the Company included herein have been prepared, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission (the "SEC"). Certain information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States of America have been condensed or omitted from this report, as is permitted by such rules and regulations. Accordingly, these condensed consolidated financial statements should be read in conjunction with the financial statements and notes thereto included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2015 (the "Annual Report").

The unaudited condensed consolidated financial statements include the accounts of Editas Medicine, Inc. and its wholly owned subsidiary. All intercompany transactions and balances of the subsidiary have been eliminated in consolidation. In the opinion of management, the information furnished reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair presentation of the results for the reported interim periods. The Company considers events or transactions that occur after the balance sheet date but before the financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The three months ended September 30, 2016 and 2015 are referred to as the third quarter of 2016 and 2015, respectively. The results of operations for interim periods are not necessarily indicative of results to be expected for the full year or any other interim period.

Use of estimates

The preparation of condensed consolidated financial statements in conformity with United States generally accepted accounting principles requires management to make estimates and assumptions that affect the amounts reported in the condensed consolidated financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, estimates related to revenue recognition, accrued expenses, stock-based compensation expense, valuation of the redeemable convertible preferred stock tranche liability and the anti-dilutive protection liability, valuation of the warrant liability, deferred tax valuation allowances, the fair value of common stock prior to the completion of the IPO, and construction lease financing obligations. The Company bases its estimates on historical experience and other market-specific or relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

Summary of significant accounting policies

The Company's significant accounting policies are described in Note 2, "Summary of Significant Accounting Policies," in the Annual Report. There have been no material changes to the significant accounting policies previously disclosed in the Annual Report.

Recent accounting pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-09, Revenue From Contracts With Customers ("ASU No. 2014-09"). ASU No. 2014-09 amends Accounting Standards Codification ("ASC") Topic 605, *Revenue Recognition*, by outlining a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers. ASU No. 2014-09 will be effective for the Company for interim and annual periods beginning after December 15, 2017. The Company is evaluating the impact that this ASU may have on its consolidated financial statements.

In August 2014, the FASB issued ASU No. 2014-15, Presentation of Financial Statements—Going Concern, which requires management to assess an entity's ability to continue as a going concern every reporting period, and provide certain disclosures if management has substantial doubt about the entity's ability to operate as a going concern, or an express statement if not, by incorporating and expanding upon certain principles that are currently in U.S. auditing standards. This guidance is effective for the annual period ending after December 15, 2016, and for annual periods and

interim periods within annual periods beginning thereafter. Early application is permitted. The Company is in process of evaluating this guidance and determining the expected effect on its consolidated financial statements, but does not expect it to have a significant impact on the Company's results of operations, cash flows or financial position.

In February 2016, the FASB issued ASU No. 2016-02, Leases (“ASU 2016-02”), which applies to all leases and will require lessees to put most leases on the balance sheet, but recognize expense in a manner similar to the current standard. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018 and interim periods within those years. Entities are required to use a modified retrospective approach of adoption for leases that exist or are entered into after the beginning of the earliest comparative period in the financial statements. Full retrospective application is prohibited. The Company is evaluating the impact that this ASU may have on its consolidated financial statements.

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

3. Fair Value Measurements

The Company classifies fair value based measurements using a three-level hierarchy that prioritizes the inputs used to measure fair value. This hierarchy requires entities to maximize the use of observable inputs and minimize the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows: Level 1, quoted market prices in active markets for identical assets or liabilities; Level 2, observable inputs other than quoted market prices included in Level 1 such as quoted market prices for markets that are not active or other inputs that are observable or can be corroborated by observable market data; and Level 3, unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities, including estimates and assumptions developed by the Company, reflective of those that a market participant would use, as inputs to certain pricing models, discounted cash flow methodologies and similar techniques that use significant unobservable inputs.

Assets measured at fair value on a recurring basis as of September 30, 2016 were as follows (in thousands):

Financial Assets:	**September 30, 2016**	**Quoted Prices in Active Markets for Identical Assets (Level 1)**	**Significant Other Observable Inputs (Level 2)**	**Significant Unobservable Inputs (Level 3)**
Cash and cash equivalents	$ 199,874	$ 199,874	$ —	$ —
Money market funds, included in other current assets	320	320	—	—
Money market funds, included in other non-current assets	1,619	1,619	—	—
Total financial assets	$ 201,813	$ 201,813	$ —	$ —

Assets and liabilities measured at fair value on a recurring basis as of December 31, 2015 were as follows (in thousands):

Financial Assets:	December 31, 2015	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents	$ 143,180	$ 143,180	$ —	$ —
Money market funds, included in other current assets	320	320	—	—
Total financial assets	$ 143,500	$ 143,500	$ —	$ —
Financial Liabilities:				
Warrant liability	$ 289	$ —	$ —	$ 289
Total financial liabilities	$ 289	$ —	$ —	$ 289

The following table sets forth a summary of the changes in the fair value of the Company's Level 3 financial liabilities (in thousands):

	Warrant Liability
Fair value at December 31, 2015	$ 289
Changes in fair value recognized in other expense	87
Reclassification to additional paid-in capital in connection with IPO	(376)
Fair value at September 30, 2016	$ —

There were no transfers between fair value measurement levels during the nine month period ended September 30, 2016 or 2015. The fair value of the preferred stock warrant liability was determined based on "Level 3" inputs utilizing the Black-Scholes option pricing model. Upon the completion of the IPO, the Company's outstanding warrant to purchase preferred stock converted into a warrant to purchase common stock and the Company reclassified the fair value of the warrant to additional paid-in capital.

Cash and cash equivalents

The Company considers all highly liquid securities with original final maturities of three months or less from the date of purchase to be cash equivalents. As of September 30, 2016 and December 31, 2015, cash and cash equivalents comprised of funds in cash and money market accounts.

4. Accrued expenses

Accrued expenses consisted of the following (in thousands):

	As of September 30, 2016	As of December 31, 2015
Patent and license fees	$ 5,762	$ 3,395
Deferred initial public offering costs	—	283
Employee compensation costs	1,930	1,016
Professional services	1,163	382
Other	637	380
Total	$ 9,492	$ 5,456

5. Property and equipment, net

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

	As of September 30, 2016	As of December 31, 2015
Construction-in-progress	$ 32,595	$ —
Laboratory equipment	4,734	2,215
Computer equipment	726	447
Furniture and office equipment	169	74
Leasehold improvements	191	23
Total property and equipment	38,415	2,759
Less: accumulated depreciation	(1,343)	(629)
Property and equipment, net	$ 37,072	$ 2,130

6. Commitments and contingencies

Facility leases

In December 2013, the Company entered into an agreement to sublease its facility under a non-cancelable operating lease that was set to expire at the end of September 2016. In August 2016, the Company amended the sublease to extend the expiration date from September 30, 2016 until the earlier of (i) 30 days following the date on which the Company gives the landlord written notice that it intends to vacate the premises and (ii) November 30, 2016. In September 2016, the Company delivered notice of its intent to vacate as of October 31, 2016, such that the sublease expired on November 1, 2016. Pursuant to the sublease agreement, the Company maintained restricted cash of $0.3 million in a collateral account that was held until the termination of the Company's obligations under the agreement. The sublease agreement could not be extended beyond the expiration date of the sublease. The sublease contained escalating rent clauses which required higher rent payments in future years. The Company expensed rent on a straight-line basis over the term of the sublease, including any rent-free periods. The deposit is recorded in prepaid expenses and other current assets in the accompanying condensed consolidated balance sheet as of September 30, 2016 and December 31, 2015.

In November 2015, the Company entered into a real estate license agreement to sublease from the licensor additional laboratory space in Cambridge, Massachusetts. The term of the lease is from December 1, 2015 to November 30, 2016. The Company's contractual obligation related to lease payments over the term of the sublease is approximately $1.9 million. The Company delivered notice of its intent to vacate these premises in September 2016, in accordance with its right to cancel the sublease upon no less than 30 days' written notice, provided that the Company remains liable to continue to pay the monthly rental fee for the remainder of the term unless the licensor can sublease the space. If the licensor can sublease the space to another party, the Company will be credited the lesser of (i) the rental fee paid by such party corresponding to the remainder of the term and (ii) 50% of the rental for the remainder of the term.

Hurley Street, Cambridge, MA

In February 2016, the Company entered into a lease agreement for approximately 59,783 square feet of office and laboratory space located on Hurley Street in Cambridge, Massachusetts. The term of the lease began on October 1, 2016. In connection with the lease and as a security deposit, the Company deposited with the landlord a letter of credit in the amount of approximately $1.6 million. Subject to the terms of the lease and certain reduction requirements specified therein, the $1.6 million security deposit may decrease over time. The letter of credit, which is collateralized by the Company with cash held in a money market account, is recorded in restricted cash and other non-current assets in the accompanying condensed consolidated financial statement as of September 30, 2016.

In connection with this lease, the landlord provided a tenant improvement allowance for costs associated with the design, engineering, and construction of tenant improvements for the leased facility. For accounting purposes, the Company was deemed the owner of the building during the construction period due to the fact that the Company was involved in the construction project, including having responsibilities for cost overruns for planned tenant improvements that did not qualify as "normal tenant improvements" under the lease accounting guidance. Throughout the construction period, the Company recorded the project construction costs incurred as an asset, along with a corresponding facility lease obligation, on its balance sheet for the total amount of the project costs incurred whether funded by the Company or the landlord. As of September 30, 2016, the Company had recorded construction in progress of $32.6 million, which was included in property and equipment, net, and a corresponding facility lease obligation of $32.6 million. The Company did not pay any cash to the landlord related to the building for the nine months ended September 30, 2016.

Construction was completed in October 2016, and the Company considered the requirements for sale-leaseback accounting treatment, which included an evaluation of whether all risks of ownership had transferred back to the landlord, as evidenced by a lack of continuing involvement in the leased property. The Company determined that the arrangement did not qualify for sale-leaseback accounting treatment, the building asset will remain on the Company's balance sheet at its historical cost, and such asset will be depreciated over its estimated useful life of 30 years.

The Company bifurcates its future lease payments pursuant to the Hurley Street lease into (i) a portion that is allocated to the building and (ii) a portion that is allocated to the land on which the building is located, which is recorded as rental expense. Although the Company did not begin making lease payments pursuant to the Hurley Street lease until November 2016, the portion of the lease obligation allocated to the land is treated for accounting purposes as an operating lease that commenced upon execution of the Hurley Street lease in February 2016. During the nine months ended September 30, 2016, the Company recognized $0.4 million of non-cash rental expense attributable to the land.

The lease will continue until October 2023. The Company has the option to extend the lease for an additional five-year term at market-based rates. The base rent is subject to increases over the term of the lease. The non-cancelable minimum annual lease payments for the annual periods beginning upon commencement of the lease are $3.9 million, $4.0 million, $4.1 million, $4.2 million and $4.3 million in the first five years of the lease, respectively, and $9.2 million in total thereafter, plus the Company's share of the facility operating expenses and other costs that are reimbursable to the landlord under the lease. The Company began using this space as its headquarters in October 2016 and rental payments for this property began in November 2016.

Licensor Expense Reimbursement

The Company is obligated to reimburse The Broad Institute Inc. ("Broad"), and the President and Fellows of Harvard College ("Harvard"), for expenses incurred by each of them associated with the prosecution and maintenance of the patent rights that the Company licenses from them pursuant to the license agreement by and among the Company, Broad and Harvard, including the interference and opposition proceedings involving patents licensed to the Company under the license agreement. As such, the Company anticipates that it has a substantial commitment in connection with these proceedings until such time as these proceedings have been resolved, but the amount of such commitment is not determinable. The Company incurred an aggregate of $16.0 million and $3.9 million in expense for such reimbursement during the nine months ended September 30, 2016 and 2015, respectively. During the three months ended September 30, 2016 and 2015, the Company recognized $5.0 million and $1.1 million in expense for such reimbursement, respectively.

Massachusetts General Hospital

In August 2016, the Company entered into an exclusive patent license agreement (the "2016 MGH Agreement") with The General Hospital Corporation, d/b/a Massachusetts General Hospital ("MGH"). Pursuant to the terms of such license, the Company is required to make certain success payments to MGH, payable in cash or common stock (the "MGH Success Payments") at the Company's election. The MGH Success Payments are payable, if and when, the Company's market capitalization reaches specified thresholds or upon a sale of the Company for consideration in excess of those thresholds, as discussed more fully in Note 7 (collectively the "Payment Conditions").

The MGH Success Payments were accounted for under the provisions of FASB ASC, Topic 505-50, *Equity-Based Payments to Non-Employees.* The Company has the right to terminate the agreement at will upon 90 days written notice to MGH. Absent any of the Payment Conditions being achieved prior to termination, the Company would not be obligated to pay any MGH Success Payments. As such, the Company will recognize the expense and liability associated with each MGH Success Payment upon achievement of the associated Payment Conditions, if ever. The Company did not incur any expenses related to the MGH Success Payments during the three or nine months ended September 30, 2016.

Litigation

The Company is not a party to any litigation and did not have contingency reserves established for any litigation liabilities as of September 30, 2016 or December 31, 2015.

7. Significant Agreements

Juno Therapeutics Collaboration Agreement

Summary of Agreement

In May 2015, the Company entered into a Collaboration and License Agreement (the "Collaboration Agreement") with Juno Therapeutics. The collaboration is focused on the research and development of engineered T cells with chimeric antigen receptors ("CARs") and T cell receptors ("TCRs") that have been genetically modified to recognize and kill other cells. The parties will pursue the research and development of CAR and TCR engineered T cell products utilizing the Company's genome editing technologies with Juno Therapeutics' CAR and TCR technologies across three research areas.

The collaborative program of research to be undertaken by the parties pursuant to the Collaboration Agreement will be conducted in accordance with a mutually agreed upon research plan which outlines each party's research and development responsibilities across the three research areas. The Company's research and development responsibilities under the research plan are related to generating genome editing reagents that modify gene targets selected by Juno Therapeutics. Juno Therapeutics is responsible for evaluating and selecting for further research and development CAR and TCR engineered T cell products modified with the Company's genome editing reagents. Except with respect to the Company's obligations under the mutually agreed upon research plan, Juno Therapeutics has sole responsibility, at its own cost, for the worldwide research, development, manufacturing and commercialization of products within each of the three research areas for the diagnosis, treatment or prevention of any cancer in humans through the use of engineered T cells, excluding the diagnosis, treatment or prevention of medullary cystic kidney disease 1 (the "Exclusive Field").

The initial term of the research program commenced on May 26, 2015 and continues for five years ending on May 26, 2020 (the "Initial Research Program Term"). Juno Therapeutics may extend the Initial Research Program Term for up to two additional one year periods upon the payment of extension fees for each one year extension period, assuming the Company has agreed to the extension request(s) (together, the initial term and any extension period(s) are referred to as the "Research Program Term").

Under the terms of the Collaboration Agreement, the Company granted to Juno Therapeutics during the Research Program Term a nonexclusive, worldwide, royalty-free, sublicensable (subject to certain conditions) license under certain of the intellectual property controlled by the Company solely for the purpose of conducting activities

required under the specified research under the Collaboration Agreement: (i) conduct activities assigned to Juno Therapeutics under the research plan, (ii) conduct activities assigned to the Company under the research plan that the Company fails or refuses to conduct in a timely manner, (iii) use certain genome editing reagents generated under the research program to research, evaluate and conduct preclinical testing and development of certain engineered T cells and (iv) evaluate the data developed in the conduct of activities under the research plan (the "Research License"). Additionally, as it relates to two of the three research areas, the Company granted to Juno Therapeutics an exclusive, milestone and royalty-bearing, sublicensable license under certain of the intellectual property controlled by the Company to research, develop, make and have made, use, offer for sale, sell, import and export selected CAR and TCR engineered T cell products in the Exclusive Field on a worldwide basis, specifically as it relates to certain targets selected by Juno Therapeutics pursuant to the research program. Furthermore, as it relates to the same two research areas, the Company granted to Juno Therapeutics a non-exclusive, milestone and royalty-bearing, sub licensable license under certain of the intellectual property controlled by the Company to use genome editing reagents generated under the research program that are used in the creation of certain CAR or TCR engineered T cell products on which Juno Therapeutics has filed an IND in the Exclusive Field for the treatment or prevention of a cancer in humans to research, develop, make and have made, use, offer for sale, sell, import and export those CAR or TCR engineered T cell products in all fields outside of the Exclusive Field (the "Non-Exclusive Field") on a worldwide basis, specifically as it relates to certain targets selected by Juno Therapeutics pursuant to the research program (together, the license in the Exclusive Field and the license in the Non-Exclusive Field are referred to as the "Development and Commercialization License" for each particular research area). Lastly, as it relates to the third research area, the Company granted to Juno Therapeutics a milestone and royalty-bearing, sublicensable license under certain of the intellectual property controlled by the Company to use the genome editing reagents generated under the research program that are associated with certain CAR or TCR engineered T cell products to research, develop, make and have made, use, offer for sale, sell, import or export those CAR or TCR engineered T cell products in the Exclusive Field on a worldwide basis, specifically as it relates to certain products selected by Juno Therapeutics pursuant to the research program. The license associated with the third research area is exclusive as it relates to CAR or TCR engineered T cell products directed to certain targets as selected by Juno Therapeutics, but is otherwise non-exclusive (referred to as the "Development and Commercialization License" for the third research area).

The Collaboration Agreement will be managed on an overall basis by a project leader from each of the Company and Juno Therapeutics. The project leaders will serve as the contact point between the parties with respect to the research program and will be primarily responsible for facilitating the flow of information, interaction, and collaboration between the parties. In addition, the activities under the Collaboration Agreement during the Research Program Term will be governed by a joint research committee ("JRC") formed by an equal number of representatives from the Company and Juno Therapeutics. The JRC will oversee, review and recommend direction of the research program. Among other responsibilities, the JRC will monitor and report research progress and ensure open and frequent exchange between the parties regarding research program activities.

Under the terms of the Collaboration Agreement, the Company received a $25.0 million up-front, non-refundable, non-creditable cash payment. In addition, Juno Therapeutics will pay to the Company an aggregate of up to $22.0 million in research and development funding over the initial five year term of the research program across the three research areas consisting primarily of funding for up to a specified maximum number of full time equivalents personnel each year over the initial five year term of the research program across three research areas. Under the terms of the Collaboration Agreement, there is no incremental compensation due to the Company with respect to the Development and Commercialization License granted to Juno Therapeutics associated with the first target or product, as applicable, designated by Juno Therapeutics within each of the three research areas. However, for two of the three research areas, Juno Therapeutics has the option to purchase up to three additional Development and Commercialization Licenses associated with other gene targets for an additional fee of approximately $2.5 million per target. In addition, Juno Therapeutics would be required to make certain milestone payments to the Company upon the achievement of specified development, regulatory and commercial events. More specifically, for the first product to achieve the associated event in each of the three research areas, the Company is eligible to receive up to a $77.5 million in development milestone payments and up to $80 million in regulatory milestone payments. In addition, the Company is eligible to receive additional development and regulatory milestone payments for subsequent products developed within each of the three research areas. Moreover, the Company is eligible for up to $75.0 million in commercial milestone payments associated with aggregate sales of all products within each of the three research areas. Development milestone payments are triggered upon the achievement of certain specified development criteria or upon initiation of a defined

phase of clinical research for a product candidate. Regulatory milestone payments are triggered upon approval to market a product candidate by the United States Food and Drug Administration ("FDA") or other global regulatory authorities. Commercial milestone payments are triggered when an approved pharmaceutical product reaches certain defined levels of net sales by the licensee.

In addition, to the extent any of the product candidates covered by the licenses conveyed to Juno Therapeutics are commercialized, the Company would be entitled to receive tiered royalty payments of low double digits based on a percentage of net sales. Royalty payments are subject to certain reductions, including for any royalty payments required to be made by Juno Therapeutics related to a third-party's intellectual property rights, subject to an aggregate minimum floor. Royalties are due on a licensed product-by-licensed product and country-by-country basis from the date of the first commercial sale of each product in a country until the later of: (i) the tenth anniversary of the first commercial sale of such licensed product in such country and (ii) the expiration date in such country of the last to expire valid claim within the licensed intellectual property covering the manufacture, use or sale of such licensed product in such country. In May 2016, the Company achieved a $2.5 million milestone under the collaboration resulting from technical progress in a research program to create engineered T cells with CARs and TCRs to treat cancer. Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with drug development, no additional milestone or royalty payments may ever be received from Juno Therapeutics. As of September 30, 2016, the next potential milestone payment that the Company may be entitled to receive under the agreement is a substantive milestone payment of $2.5 million for the achievement of certain development criteria. The Company would recognize the milestone payment as revenue upon achievement. There are no cancellation, termination or refund provisions in the Collaboration Agreement that contain material financial consequences to the Company.

Unless earlier terminated, the Collaboration Agreement will continue in full force and effect, on a product-by-product and country-by-country basis until the date no further payments are due to the Company from Juno Therapeutics. Either party may terminate the Collaboration Agreement if the other party has materially breached or defaulted in the performance of any of its material obligations and such breach or default continues after the specified cure period. Either party may terminate the Collaboration Agreement in the event of the commencement of any proceeding in or for bankruptcy, insolvency, dissolution or winding up by or against the other party that is not dismissed or otherwise disposed of within a specified time period. Juno Therapeutics may terminate the Collaboration Agreement for convenience upon not less than six months prior written notice to the Company. The Company may terminate the Collaboration Agreement in the event that Juno Therapeutics brings, assumes, or participates in, or knowingly, willfully or recklessly assists in bringing a dispute or challenge against the Company related to its intellectual property.

Termination of the Collaboration Agreement for any reason does not release either party from any liability which, at the time of such termination, has already accrued to the other party or which is attributable to a period prior to such termination nor preclude either party from pursuing any rights and remedies it may have under the agreement or at law or in equity with respect to any breach of the Collaboration Agreement. If Juno Therapeutics terminates the Collaboration Agreement as a result of the Company's uncured material breach or default, then: (i) the licenses and rights conveyed to Juno Therapeutics will continue as set forth in the agreement, (ii) Juno Therapeutics' obligations related to milestones and royalties will continue as set forth in the agreement and (iii) Juno Therapeutics' rights to prosecute, maintain and enforce certain intellectual property rights will continue as set forth in the agreement. If Juno Therapeutics terminates the Collaboration Agreement for convenience or if the Company terminates the Collaboration Agreement as a result of Juno Therapeutics' uncured material breach or default, then the licenses conveyed to Juno will terminate.

Accounting Analysis

The Company evaluated the Collaboration Agreement in accordance with the provisions of ASC, Topic 605-25, *Revenue Recognition—Multiple Element Arrangements*. The Company's arrangement with Juno Therapeutics contains the following deliverables: (i) research and development services during the Initial Research Program Term (the "R&D Services Deliverable"), (ii) the Research License, (iii) the Development and Commercialization Licenses related to each of the three research areas (each, the "Development and Commercialization License Deliverable" for the respective research area), (iv) significant and incremental discount related to the option to purchase up to three additional

Development and Commercialization Licenses for two of the research areas (each, the “Discount Deliverable” for the associated option) and (v) JRC services during the Initial Research Program Term (the “JRC Deliverable”).

The Company has determined that the options to purchase additional development and commercialization licenses within two of the research program areas related to other gene targets are substantive options. Juno Therapeutics is not contractually obligated to exercise the options. Moreover, as a result of the uncertain outcome of the discovery, research and development activities, there is significant uncertainty as to whether Juno Therapeutics will decide to exercise its option for any additional gene targets within either of the two applicable research areas. Consequently, the Company is at risk with regard to whether Juno Therapeutics will exercise the options. However, the Company has determined that the options to purchase additional development and commercialization licenses with respect to other gene targets within the two applicable research program areas are priced at a significant and incremental discount. As a result, the Company has concluded that the discounts to purchase development and commercialization licenses for up to three additional gene targets within both of the research areas represent separate elements in the arrangement at inception. Accordingly, the deliverables identified at inception of the arrangement include six separate deliverables related to the significant and incremental discount inherent in the pricing of the option to purchase up to three additional development and commercialization licenses for two of the research areas included within the research program.

The Company has concluded that the Research License deliverable does not qualify for separation from the R&D Services Deliverable. As it relates to the assessment of standalone value, the Company has determined that Juno Therapeutics cannot fully exploit the value of the Research License deliverable without receipt of the R&D Services Deliverable. This is primarily due to the fact that Juno Therapeutics must rely upon the Company to provide the research and development services included in the research plan because the services incorporate technology that is proprietary to the Company. The services to be provided by the Company involve unique skills and specialized expertise, particularly as it relates to genome editing technology that is not available in the marketplace. Accordingly, Juno Therapeutics must obtain the research and development services from the Company which significantly limits the ability for Juno Therapeutics to utilize the Research License for its intended purpose on a standalone basis. Therefore, the Research License deliverable does not have standalone value from the R&D Services Deliverable. As a result, the Research License deliverable and the R&D Services Deliverable have been combined as a single unit of accounting (the “R&D Services Unit of Accounting”). Conversely, the Company has concluded that each of the other deliverables identified at the inception of the arrangement has standalone value from each of the other elements based on their nature. Factors considered in this determination included, among other things, the capabilities of the collaboration partner, whether any other vendor sells the item separately, whether the value of the deliverable is dependent on the other elements in the arrangement, whether there are other vendors that can provide the items and if the customer could use the item for its intended purpose without the other deliverables in the arrangement. Additionally, the Collaboration Agreement does not include a general right of return. Accordingly, each of the other deliverables included in the Juno Therapeutics arrangement qualifies as a separate unit of accounting.

Therefore, the Company has identified eleven units of accounting in connection with its obligations under the collaboration arrangement with Juno Therapeutics as follows: (i) the R&D Services Unit of Accounting, (ii) three units of accounting related to the Development and Commercialization Licenses for each of the three research areas, (iii) six units of accounting related to each of the Discount Deliverables, and (iv) the JRC Deliverable.

The Company has determined that neither vendor specific objective evidence of selling price nor third-party evidence of selling price is available for any of the units of accounting identified at inception of the arrangement with Juno Therapeutics. Accordingly, the selling price of each unit of accounting was determined based on the Company’s best estimate of selling price (“BESP”). The Company developed the BESP for all of the units of accounting included in the Collaboration Agreement with the objective of determining the price at which it would sell such an item if it were to be sold regularly on a standalone basis. The Company developed the BESP for the R&D Services Unit of Accounting and the JRC Deliverable primarily based on the nature of the services to be performed and estimates of the associated effort and cost of the services, adjusted for a reasonable profit margin that would be expected to be realized under similar contracts. The Company developed the BESP for each of the Development and Commercialization License units of accounting based on the probability-weighted present value of expected future cash flows associated with each license related to each specific research area. In developing such estimate, the Company also considered applicable market conditions and relevant entity-specific factors, including those factors contemplated in negotiating the agreement,

probability of success and the time needed to commercialize a product candidate pursuant to the associated license. The Company developed the BESP for each of the Discount Deliverables based on the estimated value of the associated in-the-money options. In developing such estimate, the Company considered the period to exercise the option, an appropriate discount rate and the likelihood that a market participant who was entitled to the discount would exercise the option.

Allocable arrangement consideration at inception is comprised of: (i) the up-front payment of $25.0 million, (ii) the research support of $20.0 million and (iii) payments related to specialized materials costs of $2.0 million. The research support of $20.0 million and payments related to specialized materials costs of $2.0 million represent contingent revenue features because the Company's retention of the associated arrangement consideration is dependent upon its future performance of research support services and development of specialized materials. The aggregate allocable arrangement consideration of $47.0 million was allocated among the separate units of accounting using the relative selling price method as follows: (i) R&D Services Unit of Accounting: $16.7 million, (ii) Development and Commercialization License for the first research area: $9.3 million, (iii) Development and Commercialization License for the second research area: $15.4 million, (iv) Development and Commercialization License for the third research area: $0.2 million, (v) the first Discount Deliverable for the first research area: $0.7 million, (vi) the second Discount Deliverable for the first research area: $0.4 million, (vii) the third Discount Deliverable for the first research area: $0.2 million, (viii) the first Discount Deliverable for the second research area: $2.0 million, (ix) the second Discount Deliverable for the second research area: $1.3 million, and (x) the third Discount Deliverable for the second research area: $0.8 million. No amounts were allocated to the JRC Deliverable because the associated BESP was determined to be de minimis. The amounts allocated to each of the development and commercialization licenses are based on the respective BESP calculations, which reflect the level of risk and expected probability of success inherent in the nature of the associated research area.

The Company will recognize revenue related to amounts allocated to the R&D Services Unit of Accounting as the underlying services are performed. The Company will recognize revenue related to amounts allocated to each of the Development and Commercialization Licenses upon delivery of the associated license, assuming the research services are substantially complete at the time the license is delivered. The rights to be conveyed to Juno Therapeutics pursuant to each of the Development and Commercialization Licenses extend exclusively to an individual target or product, as applicable; therefore, delivery is deemed to occur upon the designation by Juno Therapeutics of the specific target or product, as applicable, whereupon the license becomes effective. The Company will recognize revenue related to amounts allocated to each of the Discount Deliverables upon the earlier of exercise of the associated option or upon lapsing of the underlying right, if the respective option expires unexercised.

The Company has evaluated all of the milestones that may be received in connection with the Juno Therapeutics arrangement. In evaluating if a milestone is substantive, the Company assesses whether: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (ii) the consideration relates solely to past performance, and (iii) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. All development and regulatory milestones are considered substantive on the basis of the contingent nature of the milestone, specifically reviewing factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the milestone as well as the level of effort and investment required. Accordingly, such amounts will be recognized as revenue in full in the period in which the associated milestone is achieved, assuming all other revenue recognition criteria are met. All commercial milestones will be accounted for in the same manner as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met. The Company will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

During the three months ended September 30, 2016 and 2015, the Company recognized revenue totaling approximately $0.8 million and $0.7 million, respectively, with respect to the collaboration with Juno Therapeutics. During the nine months ended September 30, 2016 and 2015, the Company recognized revenue totaling approximately $4.9 million ($2.5 million of which related to the first milestone payment) and $0.8 million, respectively, with respect to

the collaboration with Juno Therapeutics. The revenue is classified as collaboration and other research and development revenue in the accompanying condensed consolidated statement of operations. As of September 30, 2016, there was approximately $25.8 million of deferred revenue related to the Company's collaboration with Juno Therapeutics, all of which is classified as long-term in the accompanying condensed consolidated balance sheet. In addition, as of September 30, 2016, the Company has recorded accounts receivable of $1.0 million related to reimbursable research and development costs under the Collaboration Agreement for activities performed during the third quarter of 2016.

Cystic Fibrosis Foundation Therapeutics, Inc. Award Agreement

In May 2016, the Company entered into an award agreement (the "CF Award Agreement") with the Cystic Fibrosis Foundation Therapeutics, Inc. ("CFFT"), a non-profit drug discovery and development affiliate of the Cystic Fibrosis Foundation, pursuant to which it received a development award for up to $5.0 million in funding over the agreement's three year term (the "Award"). The funding from the Award is supporting the Company's cystic fibrosis development program and related technology research and development. The Company is required to contribute additional funds to the program in an amount equal to the funds contributed by CFFT under the agreement.

Pursuant to the terms of the CF Award Agreement, the Company is obligated to make royalty payments to CFFT contingent upon commercialization of an editing package, a delivery package, or a combination thereof, for modification of the cystic fibrosis transmembrane conductance regulator gene, the research or development of which was derived in whole or in part from the development program (a "CF Product"), including payments each equal to two times the amount the Company receives under the agreement, following the first commercial sale of a CF Product in the United States and the European Union, respectively. The Company is also obligated to make a payment to CFFT equal to two times the amount the Company receives under the CF Award Agreement, due in the first calendar year in which the aggregate cumulative net sales of a CF Product exceed $100 million. The payments due will not, in the aggregate, exceed ten percent of net sales of a CF Product in a year; the remaining obligation will be carried forward to subsequent year(s) until the payment of any such remaining payment does not, in the aggregate, exceed ten percent of net sales of a CF Product. The Company is also obligated to make payments to CFFT of up to two times the Award amount if the Company transfers, sells or licenses the development program technology, or if the Company enters into a change of control transaction, with such payments to be credited against the payments due upon commercialization. Following the first year anniversary of the effective date of the agreement, either party can terminate the agreement without cause by providing 90 days' notice. The Company's payment obligations survive the termination of the CF Award Agreement.

During the nine months ended September 30, 2016, the Company recognized revenue of $0.2 million with respect to the Award. The revenue is classified as collaboration and other research and development revenue in the accompanying condensed consolidated statement of operations.

Adverum Biotechnologies, Inc. Collaboration, Option, and License Agreement

In August 2016, the Company entered into an agreement with Adverum Biotechnologies, Inc. ("Adverum") to explore the delivery of genome editing medicines to treat up to five inherited retinal diseases. Under the terms of the agreement, the Company paid an upfront non-refundable fee of $1.0 million to evaluate Adverum's next generation adeno-associated viral vectors ("AAVs") for use in clinical development. The Company will support all preclinical activities related to this agreement, including research and development activities to be performed by Adverum, with $0.5 million of the upfront fee being creditable against this funding obligation. Accordingly, the Company has deferred and capitalized $0.5 million of the $1.0 million upfront fee as an advance payment for future research and development activities which the Company believes will be incurred in the future. The capitalized amount will be expensed as research and development expenses in the Company's consolidated statements of operations as the related services are performed. The Company expensed the remaining $0.5 million as research and development expense in the accompanying statement of operations during the three and nine months ended September 30, 2016.

Additionally, the Company may pay, at its discretion, an additional fee of $1.0 million, per exercise, to exercise an option to receive an exclusive license to Adverum's next generation AAVs for use in an indication chosen under the agreement. Adverum is also entitled to receive development and regulatory milestone payments up to a maximum of a mid-single digit millions of dollars per license based on the achievement of specific events for a product candidate that

includes an Adverum vector ("Adverum Product") and a low to mid-single digit millions of dollars based on the achievement of specific events for a product candidate that doesn't include an Adverum vector ("Non-Adverum Product"). Adverum is also entitled to receive certain commercial milestone payments for Adverum Products up to a maximum amount of a low double digit million dollar amount per product. The Company is also obligated to pay Adverum single digit to low double digit percentage royalties on net sales of Adverum Products and low single digit percentage royalties on sales of Non-Adverum Products sold in applicable territories during the royalty term.

Other Agreements

Licensing Agreements

The Company is a party to a number of license agreements under which the Company licenses patents, patent applications and other intellectual property from third parties. The Company anticipates entering into these types of license agreements in the future. The Company believes the following agreements are significant to the business:

The General Hospital Corporation License Agreements—In August 2014, the Company entered into an agreement to license certain patent rights owned or co-owned by MGH. Consideration for the granting of the license included the payment of an upfront license fee of $0.1 million, the issuance of 66,848 shares of the Company's common stock, which was based on 0.5% of the Company's outstanding stock on a fully diluted basis, and the right to receive future issuances of shares of common stock to maintain MGH's ownership following the third tranche of the Company's Series A redeemable convertible preferred stock financing (e.g. anti-dilution protection liability), which was settled in June 2015. MGH is entitled to nominal annual license fees and to receive future clinical, regulatory and commercial milestone payments aggregating to a maximum of $3.7 million and aggregate of $1.8 million upon the occurrence of certain sales milestones. The Company is also obligated to pay MGH low single digit percentage royalties on net sales of products for the prevention or treatment of human disease, and ranging from low single digit to low double digit percentage royalties on net sales of other products and services made by the Company, its affiliates or its sublicenses. The royalty percentage depends on the product and service, and whether such licensed product or licensed service is covered by a valid claim within the certain patent rights that the Company licenses from MGH.

In August 2016, the Company entered into the 2016 MGH Agreement to license certain patent rights owned or co-owned by MGH (the "Additional MGH Patent Rights"). Consideration for granting the license included the payment of an upfront nonrefundable license fee of $0.8 million, which the Company recorded as research and development expense in the accompanying consolidated statement of operations. Under the 2016 MGH Agreement, MGH is entitled to nominal annual license fees, clinical and regulatory milestone payments totaling less than $1.0 million in the aggregate per licensed product up to four licensed products or processes to achieve the specified clinical and regulatory milestones, and commercial sales milestone payments totaling up to $4.9 million in the aggregate, consisting of milestone payments due upon the first commercial sales for up to four licensed products or processes and milestone payments due upon annual net sales of products or processes meeting specified thresholds. The Company is also obligated to pay MGH royalties of less than 1% on net sales of products and processes for the prevention or treatment of human disease, and royalties of a low single-digit percentage on net sales of products and processes for the prevention or treatment of a non-human animal disease, made by the Company, its affiliates, or its sublicensees. The royalty percentages that the Company is obligated to pay are subject to reduction if at the time of sale the applicable product or process is not covered by a valid claim within the Additional MGH Patent Rights. Under the 2016 MGH Agreement, the Company is obligated to reimburse MGH for all patent costs and future reasonable costs associated with the prosecution, filing, and maintenance of the licensed patents.

MGH is also entitled under the 2016 MGH Agreement to receive MGH Success Payments of up to $6.0 million in the event the Company's market capitalization reaches specified thresholds exceeding a low ten digit dollar amount, on or prior to the expiration or termination of the 2016 MGH Agreement (or if earlier, a Company sale) ("Market Cap Success Payments") or a Company sale for consideration in excess of those thresholds ("Company Sale Success Payments"). Additional Market Cap Success Payments become payable, and the amount of potential Company Sale Success Payments would increase further, if the Company's market capitalization reaches additional higher thresholds and the Company has at least one product candidate that is covered by a claim of an Additional MGH Patent Right and that (i) is the subject of a Phase 1 clinical trial of which the Company or an affiliate or sublicensee of the Company is the

sponsor, (ii) was the subject of a Phase 1 clinical trial of which the Company or an affiliate or sublicensee of the Company was the sponsor with the Company having determined to conduct a subsequent clinical trial with respect to such product candidate, or (iii) has been approved for sale in either the United States or European Union. Market Cap Success Payments are payable in cash or shares of Company common stock at the Company's discretion, and Company Sale Success Payments are payable solely in cash. For additional information regarding the 2016 MGH Agreement, see Note 6 to these condensed consolidated financial statements.

The Broad Institute, Inc., The President and Fellows of Harvard College, and Massachusetts Institute of Technology License Agreement—In October 2014, the Company entered into an agreement with Harvard and Broad to license certain patent rights owned or co-owned by, or among, Harvard, Massachusetts Institute of Technology ("MIT"), and the Broad (collectively, the "Institutions"). Consideration for the granting of the license included the payment of an upfront license issuance fee of $0.2 million, the issuance of 561,531 shares of the Company's common stock, which was equal to 4.2% of the Company's outstanding stock on a fully diluted basis and, the right to receive future issuances of shares of common stock to maintain the Institutions' ownership following the third tranche of the Series A Preferred Stock financing (e.g. anti-dilution protection liability), which was settled in June 2015. The Institutions are collectively entitled to receive clinical and regulatory milestone payments totaling up to $14.8 million in the aggregate per licensed product approved in the United States, European Union, and Japan for the treatment of a human disease that afflicts at least a specified number of patients in the aggregate in the United States. If the Company undergoes a change of control during the term of the license agreement, the clinical and regulatory milestone payments will be increased by a certain percentage in the mid-double digits. The Company is also obligated to make additional payments to the Institutions, collectively, of up to an aggregate of $54.0 million upon the occurrence of certain sales milestones per licensed product for the treatment of a human disease that afflicts at least a specified number of patients in the aggregate in the United States. The Institutions are collectively entitled to receive clinical and regulatory milestone payments totaling up to $4.1 million in the aggregate per licensed product approved in the U.S. and at least one jurisdiction outside the U.S. for the treatment of a human disease based on certain criteria. The Company is also obligated to make additional payments to the Institutions, collectively, of up to an aggregate of $36.0 million upon the occurrence of certain sales milestones per licensed product for the treatment of a rare disease meeting certain criteria. The Institutions are entitled to receive from the Company nominal annual license fees and a mid-single digit percentage royalties on net sales of products for the prevention or treatment of human disease, and ranging from low single digit to high single digit percentage royalties on net sales of other products and services, made by the Company, its affiliates, or its sublicensees. The royalty percentage depends on the product and service, and whether such licensed product or licensed service is covered by a valid claim within the certain patent rights that the Company licenses from the Institutions.

Duke University License Agreement—In October 2014, the Company entered into an exclusive license agreement with Duke University ("Duke") to access intellectual property and technology related to the CRISPR/Cas9 and TALEN genome editing systems. In consideration for the granting of the license, the Company paid Duke an upfront fee of $0.1 million. Duke is entitled to receive clinical, regulatory, and commercial milestone payments totaling up to $0.6 million in the aggregate per licensed product. The Company is also obligated to pay to Duke nominal annual license fees and low single digit royalties based on annual net sales of licensed products and licensed services by the Company and its affiliates and sublicensees.

Each of the above license agreements obligates the Company to use commercially reasonable efforts to research, develop, and commercialize products for the prevention or treatment of human disease. The Company is also required to achieve certain development milestones within specific time periods. Each licensor has the right to terminate the license if the Company fails to achieve the development milestones. Each license agreement requires the Company to pay an annual license maintenance fee and reimburse the licensor for expenses associated with the prosecution and maintenance of the licensed patent rights.

The Company recorded the upfront issuance fees and the fair value of the common stock issued to the licensors as research and development expense (as the licenses do not have alternative future use) in accordance with ASC Topic 730, *Research and Development*. The anti-dilutive protection obligation was classified as a liability and was recorded at its grant date fair value on the effective date of the respective agreements with the initial fair value being recorded to research and development expense as it represented additional consideration paid to the licensor in connection with the license agreement. The anti-dilution liability was settled in June 2015.

8. Stock-based compensation

Total compensation cost recognized for all stock-based compensation awards in the condensed consolidated statements of operations and comprehensive loss was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Research and development	$ 2,619	$ 921	$ 10,021	$ 1,391
General and administrative	1,072	136	3,078	171
Total stock-compensation expense	$ 3,691	$ 1,057	$ 13,099	$ 1,562

Restricted Stock

From time to time, upon approval by the Company's board of directors, certain employees and advisors have been granted restricted shares of common stock. These shares of restricted stock are subject to repurchase rights. Accordingly, the Company has recorded the proceeds from the issuance of restricted stock as a liability in the condensed consolidated balance sheets included as a component of accrued expenses or other long term liabilities based on the scheduled vesting dates. The restricted stock liability is reclassified into stockholders' equity (deficit) as the restricted stock vests. A summary of the status of and changes in unvested restricted stock as of September 30, 2016 is as follows:

	Shares	Weighted Average Grant Date Fair Value Per Share
Unvested Restricted Common Stock as of December 31, 2015	1,596,853	$ 0.0188
Issued	—	—
Vested	(580,662)	$ 0.0162
Unvested Restricted Common Stock as of September 30, 2016	1,016,191	$ 0.0203

For the nine months ended September 30, 2016, the expense related to restricted stock awards granted to employees and non-employees was $0 and $7.0 million, respectively. For the nine months ended September 30, 2015, the expense related to restricted stock awards granted to employees and non-employees was $0 and $1.2 million, respectively.

As of September 30, 2016, the Company had no unrecognized stock-based compensation expense related to its employee unvested restricted stock awards. As of September 30, 2016, the Company had unrecognized stock-based compensation expense related to its non-employee unvested restricted stock awards of $4.1 million which is expected to be recognized over the remaining weighted average vesting period of 0.8 years.

Stock Options

Certain of the Company's stock option agreements allow for the exercise of unvested awards. During 2014, options to purchase 75,304 shares of common stock for $0.03 per share were exercised prior to their vesting. The unvested shares are subject to repurchase by the Company if the employees cease to provide service to the Company, with or without cause. As such, the Company does not treat the exercise of unvested options as a substantive exercise. The Company has recorded the proceeds from the exercise of unvested stock options as a liability in the condensed consolidated balance sheets as a component of accrued expenses or other long term liabilities based on the scheduled vesting dates. The liability for unvested common stock subject to repurchase is reclassified into stockholders' equity (deficit) as the shares vest.

The following is a summary of stock option activity for the nine months ended September 30, 2016:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2015	1,713,385	$ 6.31	9.6	$ 15,580
Granted	1,450,441	$ 21.36		
Exercised	(60,357)	$ 3.47		
Cancelled	(12,659)	$ 9.71		
Outstanding at September 30, 2016	3,090,810	$ 13.41	9.1	$ 11,639
Vested and expected to vest at September 30, 2016	3,040,552	$ 13.41	9.1	$ 11,449
Exercisable at September 30, 2016	362,474	$ 5.20	8.8	$ 3,055

The table above reflects unvested stock options as exercised on the dates that the shares are no longer subject to repurchase. The Company had 26,301 and 39,338 shares of unvested restricted common stock outstanding at September 30, 2016 and December 31, 2015, respectively, resulting from the exercise of unvested stock options.

Using the Black-Scholes option pricing model, the weighted average fair value of options granted to employees and directors during the three and nine months ended September 30, 2016 and 2015 was $15.01 and $4.71, respectively. The expense related to options granted to employees and directors was $4.0 million and $0.3 million for the nine months ended September 30, 2016 and 2015, respectively.

The fair value of each option issued to employees and directors was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Risk free interest rate	1.3 %	1.8 %	1.4 %	1.7 %
Expected dividend yield	—	—	—	—
Expected term (in years)	6.25	6.25	6.25	6.25
Expected volatility	76.5 %	79.2 %	78.9 %	80.0 %

There were 100,000 options granted to persons other than employees and directors during the nine months ended September 30, 2016. For the three and nine months ended September 30, 2016 and 2015, the fair value of each option issued to persons other than employees and directors was estimated at the date of grant using the Black-Scholes option pricing model with the weighted-average assumptions set forth in the table below:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Risk free interest rate	1.6 %	2.2 %	1.6 %	2.2 %
Expected dividend yield	—	—	—	—
Expected term (in years)	10.0	10.0	10.0	9.8
Expected volatility	76.5 %	80.0 %	76.5 %	79.6 %

As of September 30, 2016, the Company had unrecognized stock-based compensation expense related to its employee stock options of $22.8 million which the Company expects to recognize over the remaining weighted average vesting period of 3.0 years.

9. Net loss per share

Basic net loss per common share is calculated by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding during the period, without consideration for potentially

dilutive securities. Diluted net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares and potentially dilutive securities outstanding for the period determined using the treasury stock and if converted methods. Contingently issuable shares are included in the calculation of basic loss per share as of the beginning of the period in which all the necessary conditions have been satisfied. Contingently issuable shares are included in diluted loss per share based on the number of shares, if any, that would be issuable under the terms of the arrangement if the end of the reporting period was the end of the contingency period, if the results are dilutive.

For purposes of the diluted net loss per share calculation, stock options and warrants are considered to be common stock equivalents, but they were excluded from the Company's calculation of diluted net loss per share allocable to common stockholders because their inclusion would have been anti-dilutive. Therefore, basic and diluted net loss per share applicable to common stockholders was the same for all periods presented. Contingently issuable shares of common stock pursuant to the 2016 MGH Agreement (Note 6) are excluded from the calculation of basic and diluted net loss per share calculation as the Payment Conditions have not been satisfied.

Upon the closing of the IPO in February 2016, the Company sold 6,785,000 shares of common stock and issued an additional 24,929,709 shares of common stock in connection with the automatic conversion of its redeemable convertible preferred stock. The issuance of these shares resulted in a significant increase in the Company's weighted-average shares outstanding for the nine months ended September 30, 2016 when compared to the comparable prior year period and is expected to continue to impact the year-over-year comparability of the Company's net loss per share calculations for the next six months.

The following common stock equivalents were excluded from the calculation of diluted net loss per share allocable to common stockholders because their inclusion would have been anti-dilutive:

	As of September 30,	
	2016	2015
Redeemable convertible preferred stock	—	24,929,709
Warrant to purchase redeemable convertible preferred stock	—	23,076
Unvested restricted common stock	1,016,191	1,809,639
Outstanding stock options	3,090,810	1,199,371
Total	4,107,001	27,961,795

10. Related-party transactions

During the nine months ended September 30, 2016 and 2015, the Company paid a related party $1.3 million and $1.2 million in rent and facility-related fees, respectively. In addition, during the nine months ended September 30, 2015 the Company paid one of its investors $0.1 million in professional fees.

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

The following discussion and analysis of our financial condition and results of operations should be read together with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2015, which was filed with the Securities and Exchange Commission on March 30, 2016 (the "2015 10-K").

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. There are a number of important risks and uncertainties that could cause our actual results to differ materially from those indicated by forward-looking statements. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the section entitled "Risk Factors" in Part II, Item 1A that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments that we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report on Form 10-Q are made as of the date of this Quarterly Report on Form 10-Q,and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

Overview

We are a leading genome editing company dedicated to treating patients with genetically defined diseases by correcting their disease-causing genes. Our mission is to translate the promise of genome editing science into a broad class of transformative genomic medicines to benefit the greatest number of patients. To this end, we are developing a proprietary genome editing platform based on CRISPR/Cas9 technology. Our product development strategy is to target genetically defined diseases with an initial focus on debilitating illnesses where there are no approved treatments and where the genetic basis of disease is well understood. We are advancing discovery research programs, including programs to address genetic, infectious, and oncologic diseases of the liver, lung, blood, eye, and muscle. Our most advanced program is designed to address a specific genetic form of retinal degeneration called Leber Congenital Amaurosis type 10 ("LCA10"), a disease with no available therapies or potential treatments in clinical trials in either the United States or European Union. We aim to initiate a clinical trial in this program in 2017. In May 2015, we entered into a collaboration with Juno Therapeutics, Inc. ("Juno Therapeutics"), a leader in the emerging field of immuno-oncology, to develop novel engineered T cell therapies for cancer.

Since our inception in September 2013, our operations have focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio, assembling our core capabilities in genome editing, seeking to identify potential product candidates, and undertaking preclinical studies. All of our research programs are still in the preclinical or research stage of development and their risk of failure is high. We have not generated any revenue from product sales. We have funded our operations primarily through the initial public offering of our common stock ("IPO"), private placements of our preferred stock, an equipment loan, and payments received under our collaboration with Juno Therapeutics. From inception through September 30, 2016, we raised an aggregate of $294.4 million to fund our operations.

In February 2016, we completed our IPO and sold 6,785,000 shares of our common stock, including 885,000 shares of our common stock pursuant to the full exercise by the underwriters of an option to purchase additional shares, at a public offering price of $16.00 per share for an aggregate offering of approximately $108.6 million. We received

aggregate net proceeds from our IPO of approximately $97.5 million, after deducting underwriting discounts and commissions and other offering expenses payable by us.

Since inception, we have incurred significant operating losses. We incurred a net loss of $57.8 million for the nine months ended September 30, 2016. As of September 30, 2016, we had an accumulated deficit of $146.2 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and from year to year. We anticipate that our expenses will increase substantially as we continue our current research programs and our preclinical development activities; seek to identify additional research programs and additional product candidates; initiate preclinical testing and clinical trials for any product candidates we identify and develop; maintain, expand, and protect our intellectual property portfolio, including, reimbursing our licensors for such expenses related to the intellectual property that we in-license from such licensors; further develop our genome editing platform; hire additional clinical, quality control, and scientific personnel; and incur additional costs associated with operating as a public company. We do not expect to be profitable for the year ending December 31, 2016 or the foreseeable future.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from product sales for the foreseeable future. In connection with entering into our collaboration with Juno Therapeutics in May 2015, we received an upfront payment of $25.0 million, and in May 2016, we received a milestone payment under the collaboration of $2.5 million. In addition, we will receive up to $22.0 million in research support over the five years of the collaboration and across the three programs under the collaboration, subject to adjustment in accordance with the terms of the agreement, of which, we have recognized $4.1 million to date and $2.4 million during the nine month period ended September 30, 2016.

For the nine month period ended September 30, 2016, we recognized $4.9 million of collaboration revenue related to our collaboration with Juno Therapeutics, including $2.5 million recognized during the second quarter in connection with the achievement of our first milestone under the collaboration, resulting from technical progress in a research program under the collaboration. For additional information about our revenue recognition policy related to the Juno Therapeutics collaboration, see “Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Estimates—Revenue” in our 2015 10-K.

In May 2016, we entered into an award agreement with Cystic Fibrosis Foundation Therapeutics, Inc. (“CFFT”), pursuant to which CFFT has agreed to pay us up to $5.0 million over the agreement’s three year term to support our cystic fibrosis development program and related technology research and development. Under the terms of the agreement, we are required to contribute additional funds to the program in an amount equal to the funds contributed by CFFT and to pay certain amounts to CFFT upon the achievement of specified events. For the nine month period ended September 30, 2016, we recognized $0.2 million of revenue related to our agreement with CFFT.

For the foreseeable future, we expect substantially all of our revenue will be generated from our collaboration with Juno Therapeutics, our agreement with CFFT and any other collaborations or agreements we may enter into.

Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts and preclinical studies under our research programs, which include:

· employee-related expenses including salaries, benefits, and stock-based compensation expense;

- costs of funding research performed by third parties that conduct research and development and preclinical activities on our behalf;
- costs of purchasing lab supplies and non-capital equipment used in our preclinical activities and in manufacturing preclinical study materials;
- consultant fees;
- facility costs including rent, depreciation, and maintenance expenses; and
- fees for maintaining licenses under our third-party licensing agreements.

Research and development costs are expensed as incurred. At this time, we cannot reasonably estimate or know the nature, timing, and estimated costs of the efforts that will be necessary to complete the development of any product candidates we may identify and develop. This is due to the numerous risks and uncertainties associated with developing such product candidates, including the uncertainty of:

- successful completion of preclinical studies and Investigational New Drug-enabling studies;
- successful enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of the product, if and when approved, whether alone or in collaboration with others;
- acceptance of the product, if and when approved, by patients, the medical community, and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

A change in the outcome of any of these variables with respect to the development of any product candidates we may develop would significantly change the costs, timing, and viability associated with the development of that product candidate.

Other than in connection with our collaboration with Juno Therapeutics and our agreement with CFFT, we do not track research and development costs on a program-by-program basis as we have not yet identified a product candidate for advancement into clinical trials. We plan to track research and development costs for any individual development program when we identify a product candidate from the program that we believe we can advance into clinical trials.

Research and development activities are central to our business model. We expect research and development costs to increase significantly for the foreseeable future as our development programs progress, including as we continue to support the preclinical studies for our LCA10 program as well as our other research programs.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation for personnel in executive, finance, accounting, business development, legal, and human resource functions. Other significant costs include corporate facility costs not otherwise included in research and development expenses, legal fees related to patent and corporate matters, and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities and potential commercialization of any product candidates we identify and develop. These increases will include increased costs associated with the lease of a new facility for our headquarters and will likely include increased costs related to the hiring of additional personnel, and fees to outside consultants. We also anticipate increased expenses related to reimbursement of third-party patent-related expenses and increased expenses associated with being a public company, including costs for audit, legal, regulatory, and tax-related services, director and officer insurance premiums, and investor relations costs. With respect to reimbursement of third-party patent-related expenses specifically, given the ongoing nature of the interference and opposition proceedings involving the patents licensed to us under our license agreement with The Broad Institute, Inc. (“Broad”) and the President and Fellows of Harvard College (“Harvard”) (described in more detail in Part II. Other Information. Item 1A. Risk Factors—Risks Related to Our Intellectual Property—Some of our in-licensed patents are subject to priority disputes), we anticipate that our obligation to reimburse Broad and Harvard for expenses related to these proceedings during future periods will be substantial until such interference and opposition proceedings are resolved.

Other Income (Expense), Net

For the nine months ended September 30, 2016, other income (expense), net consisted primarily of interest income earned on our cash equivalents and government grant income, net of re-measurement losses associated with changes in the fair value of our liability for a warrant to purchase preferred stock. Upon the completion of our IPO, our outstanding warrant to purchase preferred stock converted into a warrant to purchase common stock and we reclassified the fair value of the warrant to additional paid-in capital. As a result, we ceased recognizing further re-measurement gains or losses associated with the warrant after the first quarter of 2016.

For the nine months ended September 30, 2015, other income (expense), net consisted primarily of re-measurement losses associated with changes in the fair value of tranche rights associated with our Series A-1 preferred stock, warrant liability associated with the warrant we issued to our equipment loan lender, and the anti-dilutive protection liability associated with our issuance of common stock to certain licensors. Other expenses also included interest expense and the amortization of deferred financing costs associated with our equipment loan. As a result of the settlement of the tranche rights and anti-dilutive protection rights in June 2015, we ceased recognizing re-measurement gains and losses associated with those rights after the second quarter of 2015.

Critical Accounting Policies and Estimates

Our management’s discussion and analysis of our financial condition and results of operations is based on our condensed consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles. The preparation of our condensed consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues, and expenses, and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events, and various other factors that we believe to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts, and experience. The effects of material revisions in estimates, if any, will be reflected in the condensed consolidated financial statements prospectively from the date of change in estimates.

In the first quarter of 2016, we began recording certain estimated construction costs incurred and reported to us by a landlord as an asset and corresponding construction financing lease obligation on our condensed consolidated balance sheets. Construction was completed in October 2016 and we considered the requirements for sale-leaseback accounting treatment, which included an evaluation of whether all risks of ownership had transferred back to the landlord as evidenced by a lack of continuing involvement in the leased property. We determined that the arrangement did not qualify for sale-leaseback accounting treatment, the building asset would remain on our balance sheet at its historical cost, and such asset would be depreciated over its estimated useful life. For additional information, see Note 6 to the condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

In August 2016, we entered into an exclusive patent license agreement (the "2016 MGH Agreement") with The General Hospital Corporation, d/b/a Massachusetts General Hospital ("MGH"), pursuant to which we granted certain share-based success payment rights to MGH. Pursuant to the terms of such license, we are required to make certain success payments to MGH, payable in cash or common stock, if and when, our market capitalization reaches specified thresholds or upon a sale of our company for consideration in excess of those thresholds, subject to certain limitations (the "MGH Success Payments"). For additional information regarding the 2016 MGH Agreement, see Notes 6 and 7 to the condensed consolidated financial statements included in this Quarterly Report on Form 10-Q. The MGH Success Payments were accounted for under the provisions of Financial Accounting Standards Board Accounting Standards Codification ("ASC"), Topic 505-50, *Equity-Based Payments to Non-Employees*, and a liability and related expense will be recorded only upon the achievement of the market capitalization thresholds or a sale of our business for consideration in excess of such thresholds.

There have been no material changes to our critical accounting policies from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our 2015 10-K.

Results of Operations

Comparison of the Three Months ended September 30, 2016 and 2015

The following table summarizes our results of operations for the three months ended September 30, 2016 and 2015, together with the changes in those items in dollars (in thousands):

	Three Months Ended September 30,		
	2016	2015	Dollar Change
Collaboration and other research and development revenues	$ 962	$ 670	$ 292
Operating expenses:			
Research and development	10,832	3,850	6,982
General and administrative	11,295	4,202	7,093
Total operating expenses	22,127	8,052	14,075
Other income (expense), net			
Other income, net	3	21	(18)
Interest income (expense), net	142	(44)	186
Total other income (expense), net	145	(23)	168
Net loss	$ (21,020)	$ (7,405)	$ (13,615)

Collaboration and other research and development revenues

Collaboration and other research and development revenues were $1.0 million for the three months ended September 30, 2016 and consisted of $0.8 million of revenue recognized pursuant to our collaboration with Juno Therapeutics and $0.2 million of revenue recognized pursuant to our agreement with CFFT. Collaboration and other research and development revenues were $0.7 million for the three months ended September 30, 2015 and consisted of revenue recognized pursuant to our collaboration with Juno Therapeutics.

Research and Development Expenses

Research and development expenses increased by $6.9 million, to $10.8 million for the three months ended September 30, 2016 from $3.9 million for the three months ended September 30, 2015. The $6.9 million increase was due to a $2.8 million increase in employee and non-employee related expenses, including stock-based compensation resulting from an increase in the size of our workforce, a $2.2 million increase in our process and platform development expenses due to increased research activity, a $1.1 million increase in facility-related costs as a result of additional office and laboratory space, and an increase of $0.8 million for certain license fees and expenses.

The following table summarizes our research and development expenses for the three months ended September 30, 2016 and September 30, 2015, together with the changes in those items in dollars (in thousands):

	Three Months Ended September 30,		
	2016	2015	Dollar Change
Employee and non-employee related expenses	$ 5,243	$ 2,394	$ 2,849
Process and platform development expenses	3,223	1,020	2,203
License fees and expenses	763	12	751
Facility expenses	1,547	398	1,149
Other expenses	56	26	30
Total research and development expenses	$ 10,832	$ 3,850	$ 6,982

General and Administrative Expenses

General and administrative expenses increased by $7.1 million to $11.3 million for the three months ended September 30, 2016 from $4.2 million for the three months ended September 30, 2015. The $7.1 million increase in general and administrative expenses consisted of increases of $4.3 million in legal fees to support patents that we own or in-license, including costs for the prosecution and maintenance of patents that we own or in-license as well as to procure the application for and issuance of additional patents in the United States and other jurisdictions, $1.8 million in employee compensation cost, and $1.0 million in office and facility costs related to our new headquarters.

Other income (expense), net

For the three months ended September 30, 2016, other income (expense), net consisted primarily interest income earned on our cash equivalents and government grant income.

For the three months ended September 30, 2015, other income (expense), net consisted primarily of re-measurement losses associated with changes in the fair value of the warrant liability associated with the preferred stock warrant we issued to our equipment loan lender and interest expense and amortization of deferred financing costs associated with our equipment loan, net of interest income earned on our cash equivalents and government grant income. Upon the completion of our IPO, our outstanding warrant to purchase preferred stock converted into a warrant to purchase common stock and we reclassified the fair value of the warrant to additional paid-in capital. As a result, we ceased recognizing further re-measurement gains or losses associated with the warrant after the first quarter of 2016.

Comparison of the Nine Months ended September 30, 2016 and 2015

The following table summarizes our results of operations for the nine months ended September 30, 2016 and 2015, together with the changes in those items in dollars (in thousands):

	Nine Months Ended September 30,		
	2016	2015	Dollar Change
Collaboration and other research and development revenues	$ 5,155	$ 837	$ 4,318
Operating expenses:			
Research and development	30,144	13,020	17,124
General and administrative	33,215	10,756	22,459
Total operating expenses	63,359	23,776	39,583
Other income (expense), net			
Other expense, net	(22)	(37,219)	37,197
Interest income (expense), net	419	(109)	528
Total other income (expense), net	397	(37,328)	37,725
Net loss	$ (57,807)	$ (60,267)	$ 2,460

Collaboration and other research and development revenues

Collaboration and other research and development revenues were $5.2 million for the nine months ended September 30, 2016 and consisted of $4.9 million of revenue recognized pursuant to our collaboration with Juno Therapeutics, including $2.5 million of which related to the first milestone payment, and $0.2 million of revenue recognized pursuant to our agreement with CFFT. Collaboration and other research and development revenues were $0.8 million for the nine months ended September 30, 2015 and consisted of revenue recognized pursuant to our collaboration with Juno Therapeutics.

Research and Development Expenses

Research and development expenses increased by $17.1 million to $30.1 million for the nine months ended September 30, 2016 from $13.0 million for the nine months ended September 30, 2015. The $17.1 million increase consisted primarily of a $12.4 million increase in employee and non-employee related expenses, including stock-based compensation resulting from an increase in the size of our workforce, a $5.2 million increase in our process and platform development expenses due to increased research activity, a $2.7 million increase in facility related costs as a result of additional office and laboratory space, and an increase of $0.8 million for certain license fees and expenses. These increases were partially offset from having incurred $4.1 million in fees and expenses under agreements with licensors in the nine months ended September 30, 2015 as a result of our entry into our collaboration agreement with Juno Therapeutics.

The following table summarizes our research and development expenses for the nine months ended September 30, 2016 and September 30, 2015, together with the changes in those items in dollars (in thousands):

	Nine Months Ended September 30,		
	2016	2015	Dollar Change
Employee and non-employee related expenses	$ 17,224	$ 4,821	$ 12,403
Process and platform development expenses	7,494	2,321	5,173
License fees and expenses	1,200	4,600	(3,400)
Facility expenses	3,883	1,179	2,704
Other expenses	343	99	244
Total research and development expenses	$ 30,144	$ 13,020	$ 17,124

General and Administrative Expenses

General and administrative expenses increased by $22.4 million, to $33.2 million for the nine months ended September 30, 2016 from $10.8 million for the nine months ended September 30, 2015. The $22.4 million increase in general and administrative expenses consisted of increases of $13.0 million in legal fees to support patents that we own or in-license, including costs for the prosecution and maintenance of patents that we own or in-license as well as to procure the application for and issuance of additional patents in the United States and other jurisdictions, $5.6 million in employee compensation cost, $1.1 million in consulting fees, $0.9 million in office and facility costs related to our new headquarters, $0.9 million in legal fees incurred for corporate matters and $0.9 million in other general and administrative expenses.

Other income (expense), net

For the nine months ended September 30, 2016, other income (expense), net consisted primarily of interest income earned on our cash equivalents and government grant income, net of re-measurement losses associated with changes in the fair value of our liability for a warrant to purchase preferred stock. Upon the completion of our IPO, our outstanding warrant to purchase preferred stock converted into a warrant to purchase common stock and we reclassified the fair value of the warrant to additional paid-in capital. As a result, we ceased recognizing further re-measurement gains or losses associated with the warrant after the first quarter of 2016.

For the nine months ended September 30, 2015, other income (expense), net consisted primarily of re-measurement losses associated with changes in the fair value of tranche rights associated with our Series A-1 preferred stock, warrant liability associated with the warrant we issued to our equipment loan lender, and the anti-dilutive protection liability associated with our issuance of common stock to certain licensors. Other expenses also included interest expense and the amortization of deferred financing costs associated with our equipment loan. As a result of the settlement of the tranche rights and anti-dilutive protection rights in June 2015, we ceased recognizing re-measurement gains and losses associated with those rights after the second quarter of 2015.

Liquidity and Capital Resources

Sources of Liquidity

From inception through September 30, 2016, we funded our operations primarily through proceeds from private placements of our preferred stock of $163.3 million, net proceeds of $97.5 million from our IPO, an up-front payment, research and development payments and a milestone payment under our collaboration with Juno Therapeutics of $25.0 million, $4.1 million and $2.5 million, respectively, and $2.0 million of gross proceeds from an equipment loan financing. As of September 30, 2016, we had cash and cash equivalents of $199.9 million.

Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2016 and 2015 (in thousands):

	Nine Months Ended September 30,	
	2016	2015
Net cash provided by (used in):		
Operating activities	$ (36,569)	$ 2,565
Investing activities	(4,436)	(1,030)
Financing activities	97,699	143,143
Net increase in cash and cash equivalents	$ 56,694	$ 144,678

Net Cash Used in Operating Activities

The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in components of working capital.

Net cash used in operating activities was $36.6 million for the nine months ended September 30, 2016 compared to $2.6 million of net cash provided by operating activities for the nine months ended September 30, 2015. The increase of $39.2 million in cash used in operating activities from the 2015 period compared to the 2016 period was primarily due to a decrease of $37.2 million in non-cash expense from mark-to-market of our preferred stock tranche liability and anti-dilutive protection liability; a decrease of $24.7 million in cash flows attributable to deferred revenue due to the receipt of the $25.0 million upfront payment under our collaboration with Juno Therapeutics during the nine months ended September 30, 2015; and a decrease of $1.5 million in cash flows attributable to prepaid expenses and other current assets. These decreases were partially offset by an increase of $11.5 million in stock-based compensation expense; an increase of $6.4 million in cash flows attributable to accounts payable and accrued expenses; a decrease in net loss of $2.5 million; an increase of $2.2 million in cash flows attributable to other non-current assets; and a decrease in cash used attributable to accounts receivable of $0.9 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $4.4 million for the nine months ended September 30, 2016 compared to $1.0 million for the nine months ended September 30, 2015. The increase in cash used in investing activities was primarily attributable to an increase in expenditures for the acquisition of property, plant and equipment and an increase in restricted cash related to our letter of credit for our new facility lease at Hurley Street.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $97.7 million for the nine months ended September 30, 2016, compared to $143.1 million for the nine months ended September 30, 2015. The decrease of $45.4 million was primarily related to our having received during the nine months ended September 30, 2015 $141.7 million in proceeds from the issuance of Series A-2 preferred stock and $1.5 million in proceeds received from an equipment loan, in each case net of issuance costs. The absence of these proceeds was partially offset by the proceeds received from our IPO, net of issuance costs, during the nine months ended September 30, 2016.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we further advance our current research programs and our preclinical development activities; seek to identify product candidates and additional research programs; initiate preclinical testing and clinical trials for any product candidates we identify and develop; maintain, expand, and protect our intellectual property portfolio, including, reimbursing our licensors for such expenses related to the intellectual property that we in-license from such licensors; hire additional clinical, quality

control, and scientific personnel; and incur additional costs associated with operating as a public company. In addition, if we obtain marketing approval for any product candidate that we identify and develop, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing, and distribution to the extent that such sales, marketing, and distribution are not the responsibility of a collaborator. We do not expect to generate significant recurring revenue unless and until we obtain regulatory approval for and commercialize a product candidate. Furthermore, following the closing of our IPO, we have begun to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce, or eliminate our research and development programs or future commercialization efforts.

We expect that our existing cash and cash equivalents at September 30, 2016, anticipated interest income, anticipated research support under our collaboration agreement with Juno Therapeutics, and anticipated payments from CFFT, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months. We have based our estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of drug discovery, preclinical development, laboratory testing, and clinical trials for the product candidates we may develop;
- the costs of preparing, filing, and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights, and defending intellectual property-related claims;
- the costs, timing, and outcome of regulatory review of the product candidates we may develop;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, for any product candidates for which we receive regulatory approval;
- the success of our collaboration with Juno Therapeutics;
- whether Juno Therapeutics exercises either or both of its options to extend the research program term under our collaboration (each of which would trigger an extension payment to us);
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other medicines and technologies;
- the costs of reimbursing our licensors for the prosecution and maintenance of the patent rights in-licensed by us; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive, and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, any product candidate that we identify and develop, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of genomic medicines that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing, if available, may involve agreements that

include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends.

If we raise funds through additional collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce, or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations

During the three months ended September 30, 2016, we entered into an exclusive patent license agreement with MGH under which we may become obligated to make specified payments.

Under our exclusive patent license agreement with MGH, we are required to pay nominal annual license fees and make clinical and regulatory milestone payments totaling less than $1 million in the aggregate for up to four licensed products or processes upon achievement of specified clinical and regulatory milestones and commercial sales milestone payments totaling up to $4.9 million in the aggregate upon the achievement of milestones relating to the first commercial sales of up to four licensed products or processes, as well as milestones relating to annual net sales of products or processes meeting specified thresholds. We are also obligated to reimburse MGH for all patent costs and future reasonable costs associated with the prosecution, filing, and maintenance of the licensed patents. Under the MGH agreement, we are also required to make MGH Success Payments of up to $6.0 million in the event our market capitalization reaches specified thresholds exceeding a low ten digit dollar amount, on or prior to the expiration or termination of the agreement (or if earlier, a sale of our company) ("Market Cap Success Payments") or a sale of our company for consideration in excess of those thresholds ("Company Sale Success Payments"). Additional Market Cap Success Payments become payable, and the amount of potential Company Sale Success Payments would increase further, if our market capitalization reaches additional higher thresholds and other specified clinical or commercial criteria have been achieved.

We are also obligated to pay MGH royalties of less than 1% on net sales of products and processes for the prevention or treatment of human disease, and royalties of a low single-digit percentage on net sales of products and processes for the prevention or treatment of a non-human animal disease.

Other than described above, during the three months ended September 30, 2016, there were no material changes to our contractual obligations and commitments described under Management's Discussion and Analysis of Financial Condition and Results of Operations in our Quarterly Report on Form 10-Q for the quarter ending March 31, 2016, which was filed with the Securities and Exchange Commission on May 13, 2016.

Off-Balance Sheet Arrangements

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable Securities and Exchange Commission rules.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related to changes in interest rates. As of September 30, 2016, we had cash and cash equivalents of $199.9 million, primarily held in money market mutual funds consisting of U.S. government-backed securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash equivalents, are in the form, or may be in the form of, money market funds or marketable securities and are or may be invested in U.S. Treasury and U.S. government agency obligations. Due to the short-term maturities and low risk profiles of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our investments.

While we contract with certain vendors and institutions internationally, substantially all of our total liabilities as of September 30, 2016 were denominated in the United States dollar and we believe that we do not have any material exposure to foreign currency exchange rate risk.

Inflation would generally affect us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the nine months ended September 30, 2016.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2016. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934 (the "Exchange Act"), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by the company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. 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 our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2016, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting that occurred during the period covered by this Quarterly Report on Form 10-Q that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings.

During the quarter ended September 30, 2016, there were no material developments in the legal proceedings described under Part II, Item 1. Legal Proceedings in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, which was filed with the Securities and Exchange Commission on August 10, 2016.

Item 1A. Risk Factors.

Our business is subject to numerous risks. The following important factors, among others, could cause our actual results to differ materially from those expressed in forward-looking statements made by us or on our behalf in this Quarterly Report on Form 10-Q and other filings with the Securities and Exchange Commission (the "SEC"), press releases, communications with investors, and oral statements. Actual future results may differ materially from those anticipated in our forward-looking statements. We undertake no obligation to update any forward-looking statements, whether as a result of new information, future events, or otherwise.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. Our net losses were $72.9 million, $13.7 million, and $1.8 million for the years ended December 31, 2015 and 2014 and the period ended December 31, 2013, respectively. Our net loss was $57.8 million for the nine months ended September 30, 2016. As of September 30, 2016, we had an accumulated deficit of $146.2 million. We have financed our operations primarily through the public offering of our common stock, private placements of our preferred stock, an equipment loan, our collaboration with Juno Therapeutics and our agreement with Cystic Fibrosis Foundation Therapeutics, Inc. ("CFFT"). We have devoted all of our efforts to research and development. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- continue our current research programs and our preclinical development of product candidates from our current research programs;
- seek to identify additional research programs and additional product candidates;
- initiate preclinical testing and clinical trials for any product candidates we identify and develop;
- maintain, expand, and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- seek marketing approvals for any of our product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing, and distribution infrastructure to commercialize any medicines for which we may obtain marketing approval;
- further develop our genome editing platform;
- hire additional clinical, quality control, and scientific personnel;
- add operational, financial, and management information systems and personnel, including personnel to support our product development;
- acquire or in-license other medicines and technologies;
- validate a commercial-scale current Good Manufacturing Practices ("cGMP") manufacturing facility; and
- operate as a public company.

We have not initiated clinical development of any product candidate and expect that it will be many years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must develop and eventually commercialize a medicine or medicines with significant market potential. This will require us to be successful in a range of challenging activities, including identifying product candidates, completing preclinical testing and clinical trials of product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing, and selling those medicines for which we may obtain marketing approval, and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we do, may never generate revenues that are significant or large enough to achieve profitability. We are currently only in the preclinical testing stages for our most advanced research programs. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business, or continue our operations. A decline in the value of our company could cause our stockholders to lose all or part of their investments in us.

We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce, or eliminate our research and product development programs or commercialization efforts.

We expect our expenses to increase in connection with our ongoing activities, particularly as we identify, continue the research and development of, initiate clinical trials of, and seek marketing approval for, product candidates. In addition, if we obtain marketing approval for any product candidates we may develop, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing, and distribution to the extent that such sales, marketing, manufacturing, and distribution are not the responsibility of a collaborator. In addition, relative to previous years, when we were a private company, we expect to incur significant additional costs associated with operating as a public company in 2016 and future years. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce, or eliminate our research and product development programs or future commercialization efforts.

We expect that our existing cash and cash equivalents at September 30, 2016, anticipated interest income, anticipated research support under our collaboration agreement with Juno Therapeutics, and anticipated payments from CFFT, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months. Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of drug discovery, preclinical development, laboratory testing, and clinical trials for the product candidates we may develop;
- the costs of preparing, filing, and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights, and defending intellectual property-related claims;
- the costs, timing, and outcome of regulatory review of the product candidates we may develop;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, for any product candidates for which we receive regulatory approval;
- the success of our collaboration with Juno Therapeutics;
- whether Juno Therapeutics exercises either or both of its options to extend the research program term under our collaboration (each of which would trigger an extension payment to us);
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other medicines and technologies;

- the costs of reimbursing our licensors for the prosecution and maintenance of the patent rights in-licensed by us; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive, and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully identify and develop product candidates and those are approved, we may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of medicines that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. We do not have any committed external source of funds, other than our collaboration with Juno Therapeutics and our agreement with CFFT, each of which is limited in scope and duration. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders may be materially diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends.

If we raise funds through additional collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates, or we may have to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce, or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Our short operating history may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.

We are an early-stage company. We were founded and commenced operations in the second half of 2013. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates, and undertaking preclinical studies. All of our research programs are still in the preclinical or research stage of development, and their risk of failure is high. We have not yet demonstrated an ability to initiate or successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial-scale medicine, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes about 10 to 15 years to develop a new medicine from the time it is discovered to when it is available for treating patients. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays, and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect that our financial condition and operating results will continue to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control. Accordingly, our

stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

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

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, product candidates we may identify for development. We do not anticipate generating revenues from product sales for the next several years, if ever. Our ability to generate future revenues from product sales depends heavily on our, or our collaborators', ability to successfully:

- identify product candidates and complete research and preclinical and clinical development of any product candidates we may identify;
- seek and obtain regulatory and marketing approvals for any of our product candidates for which we complete clinical trials;
- launch and commercialize any of our product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing, and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualify for adequate coverage and reimbursement by government and third-party payors for any our product candidates for which we obtain regulatory and marketing approval;
- develop, maintain, and enhance a sustainable, scalable, reproducible, and transferable manufacturing process for the product candidates we may develop;
- establish and maintain supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for any of our product candidates for which we obtain regulatory and marketing approval;
- obtain market acceptance of any product candidates we may develop as viable treatment options;
- address competing technological and market developments;
- implement internal systems and infrastructure, as needed;
- negotiate favorable terms in any collaboration, licensing, or other arrangements into which we may enter and performing our obligations in such collaborations;
- maintain, protect, and expand our portfolio of intellectual property rights, including patents, trade secrets, and know-how;
- avoid and defend against third-party interference or infringement claims; and
- attract, hire, and retain qualified personnel.

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

Risks Related to Discovery, Development, and Commercialization

We intend to identify and develop product candidates based on a novel genome editing technology, which makes it difficult to predict the time and cost of product candidate development. No products that utilize genome editing technology have been approved in the United States or in Europe, and there have only been a limited number of human clinical trials of a genome editing product candidate. Moreover, none of those trials has involved CRISPR/Cas9 technology.

We have concentrated our research and development efforts on our genome editing platform, which uses CRISPR/Cas9 technology. Our future success depends on the successful development of this novel genome editing therapeutic approach. To date, no product that utilizes genome editing has been approved in the United States or Europe. There have been a limited number of clinical trials of genome editing technologies, however no product candidates have been approved, and none of these clinical trials involved product candidates that utilize CRISPR/Cas9 genome editing technology. We are aware of at least one group in the United States and at least one group in China that are seeking to initiate clinical studies using CRISPR/Cas9 in the United States and China, respectively. Because our programs are all in the research or preclinical stage, we have not yet been able to assess safety in humans, and there may be long-term effects from treatment with any of our future product candidates that we cannot predict at this time. Any product candidates we may develop will act at the level of DNA, and, because animal DNA differs from human DNA, it will be difficult for us to test our future product candidates in animal models for either safety or efficacy. Also, animal models do not exist for some of the diseases we expect to pursue in our programs. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our genome editing platform, or any similar or competitive genome editing platforms, will result in the identification, development, and regulatory approval of any medicines. There can be no assurance that any development problems we experience in the future related to our genome editing platform or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible, and scalable manufacturing process or transferring that process to commercial partners. Any of these factors may prevent us from completing our preclinical studies or any clinical trials that we may initiate or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

Because genome editing is novel and the regulatory landscape that will govern any product candidates we may develop is uncertain and may change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

The regulatory requirements that will govern any novel genome editing product candidates we develop are not entirely clear and may change. Within the broader genome medicine field, we are aware of at least two gene therapy products that have received marketing authorization from the European Commission, and no gene therapy products have received marketing approval in the United States. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research (the “CBER”) to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee (“IBC”), a local institutional committee that reviews and oversees basic and clinical research conducted at the institution participating in the clinical trial. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the United States National Institutes of Health (the “NIH”) are also subject to review by the NIH Office of Biotechnology Activities’ Recombinant DNA Advisory Committee. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation. The same applies in the European Union. The EMA’s Committee for Advanced Therapies (the “CAT”) is responsible for assessing the quality, safety, and efficacy of advanced-therapy medicinal products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a

gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant European Union guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any CRISPR/Cas9 product candidates we may develop, but that remains uncertain at this point.

Adverse developments in clinical trials conducted by others of gene therapy products, cell therapy products, or products developed through the application of a CRISPR/Cas9 or other genome editing technology may cause the FDA, the EMA, and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing genome editing technologies, either of which could materially harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing genome editing technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop.

Adverse public perception of genomic medicines, and genome editing in particular, may negatively impact regulatory approval of, or demand for, our potential products.

Our potential therapeutic products involve editing the human genome. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of genome editing therapy for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that genome editing is unsafe, unethical, or immoral, and, consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

In addition, genome editing technology is subject to public debate and heightened regulatory scrutiny due to ethical concerns relating to the application of genome editing technology to human embryos or the human germline. For example, in April 2015, Chinese scientists reported on their attempts to edit the genome of human embryos to modify the gene for hemoglobin beta. This is the gene in which a mutation occurs in patients with the inherited blood disorder beta thalassemia. Although this research was purposefully conducted in embryos that were not viable, the work prompted calls for a moratorium or other types of restrictions on genome editing of human eggs, sperm, and embryos. The Alliance for Regenerative Medicine in Washington has called for a voluntary moratorium on the use of genome editing technologies, including CRISPR/Cas9, in research that involved altering human embryos or human germline cells. Similarly, the NIH has announced that it would not fund any use of genome editing technologies in human embryos, noting that there are multiple existing legislative and regulatory prohibitions against such work, including the Dickey-Wicker Amendment, which prohibits the use of appropriated funds for the creation of human embryos for research purposes or for research in which human embryos are destroyed. Laws in the United Kingdom prohibit genetically modified embryos from being implanted into women, but embryos can be altered in research labs under

license from the Human Fertilisation and Embryology Authority. Research on embryos is more tightly controlled in many other European countries.

Moreover, an annual worldwide threat assessment report delivered to the U.S. Congress in February 2016, the U.S. Director of National Intelligence stated that research into genome editing probably increases the risk of the creation of potentially harmful biological agents or products, including weapons of mass destruction. He noted that the broad distribution, low cost, and accelerated pace of development of genome editing technology could result in the deliberate or unintentional misuse of such technology.

Although we do not use our technologies to edit human embryos or the human germline, such public debate about the use of genome editing technologies in human embryos and heightened regulatory scrutiny could prevent or delay our development of product candidates. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair our development and commercialization of product candidates or demand for any products we may develop. Adverse events in our preclinical studies or clinical trials or those of our competitors or of academic researchers utilizing genome editing technologies, even if not ultimately attributable to product candidates we may identify and develop, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of potential product candidates we may identify and develop, stricter labeling requirements for those product candidates that are approved, and a decrease in demand for any such product candidates. Use of genome editing technology by a third party or government to develop biological agents or products that threaten the United States' national security could similarly result in such negative impacts to us.

We may not be successful in our efforts to identify, develop, or commercialize potential product candidates.

The success of our business depends primarily upon our ability to identify, develop, and commercialize products based on our genome editing platform. All of our product development programs are still in the preclinical or research stage of development. Our research programs, including those subject to our collaboration with Juno Therapeutics and our agreement with CFFT, may fail to identify potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates, or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products impractical to manufacture, unmarketable, or unlikely to receive marketing approval.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business, financial condition, results of operations, and prospects. 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.

The genome editing field is relatively new and is evolving rapidly. We are focusing our research and development efforts on CRISPR/Cas9, but other genome editing technologies may be discovered that provide significant advantages over CRISPR/Cas9, which could materially harm our business.

To date, we have focused our efforts on genome editing technologies using CRISPR/Cas9. Other companies have previously undertaken research and development of genome editing technologies using zinc finger nucleases, engineered meganucleases, and transcription activator-like effector nucleases ("TALENs"), but to date none has obtained marketing approval for a product candidate. There can be no certainty that the CRISPR/Cas9 technology will lead to the development of genomic medicines or that other genome editing technologies will not be considered better or more attractive for the development of medicines. For example, researchers, including Feng Zhang, Ph.D., one of our founders, published a manuscript regarding the discovery of a CRISPR system involving a different protein, Cpf1, which can also edit human DNA. These researchers have asserted that Cpf1 may work better than Cas9 in some cases. Cas9 may be determined to be less attractive than Cpf1 or other CRISPR proteins that have yet to be discovered. Similarly, a new genome editing technology that has not been discovered yet may be determined to be more attractive than CRISPR. Moreover, if we decide to develop genome technologies other than CRISPR/Cas9, we cannot be certain we will be able to obtain rights to such technologies. Although all of our founders who currently provide consulting and advisory

services to us in the areas of Cas9 and TALEN genome editing technologies have assignment of inventions obligations to us with respect to the services they perform for us, these assignment of inventions obligations are subject to limitations and do not extend to their work in other fields or to the intellectual property arising from their employment with their respective academic and research institutions. To obtain intellectual property rights assigned by these founders to such institutions, we would need to enter into license agreements with such institutions. For example, we do not have rights to Cpf1, and, if we were to seek such rights, there can be no assurance we could obtain such rights on commercially reasonable terms, or at all. Any of these factors could reduce or eliminate our commercial opportunity, and could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We depend heavily on the success of our most advanced program. All of our product development programs are at the preclinical or research stage. Preclinical testing and clinical trials of product candidates may not be successful. If we are unable to commercialize any product candidates we may develop or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the identification of our most advanced product development program for the treatment of Leber Congenital Amaurosis ("LCA"), type 10 ("LCA10"). Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of a product candidate for the treatment of LCA10 and other product candidates that we may identify in the future. The success of product candidates we may identify and develop will depend on many factors, including the following:

- sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials for our most advanced program;
- successful completion of preclinical studies and IND-enabling studies;
- successful enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity for our medicines;
- launching commercial sales of the medicines, if and when approved, whether alone or in collaboration with others;
- acceptance of the medicines, if and when approved, by patients, the medical community, and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile of the medicines following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates we may develop, which would materially harm our business.

Of the large number of biologics and drugs in development in the pharmaceutical industry, only a small percentage result in the submission of a Biologics License Application ("BLA") to the FDA or a marketing authorization application ("MAA") to the EMA. Not all BLAs or MAAs that are submitted to a regulatory agency are approved for commercialization. Furthermore, even if we do receive regulatory approval to market any product candidates that we may identify and develop, any such approval may be subject to limitations on the indicated uses for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our research programs, we cannot assure you that we will successfully develop or commercialize our most advanced program, or any of our other research programs. If we or any of our future development partners are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize, any product candidates we may identify and develop, we may not be able to generate sufficient revenue to continue our business.

If serious adverse events, undesirable side effects, or unexpected characteristics are identified during the development of any product candidates we may develop, we may need to abandon or limit our further clinical development of those product candidates.

We have not evaluated any product candidates in human clinical trials, and many of our proposed delivery modes have never been evaluated in human clinical trials. Moreover, we are not aware of any clinical trials involving CRISPR/Cas9 technology. It is impossible to predict when or if any product candidates we may develop will prove safe in humans. In the genomic medicine field, there have been several significant adverse events from gene therapy treatments in the past, including reported cases of leukemia and death. There can be no assurance that genome editing technologies will not cause undesirable side effects.

A significant risk in any genome editing product is that the edit will be "off-target" and cause serious adverse events, undesirable side effects, or unexpected characteristics. For example, off-target cuts could lead to disruption of a gene or a genetic regulatory sequence at an unintended site in the DNA, or, in those instances where we also provide a segment of DNA to serve as a repair template, it is possible that following off-target cut events, DNA from such repair template could be integrated into the genome at an unintended site, potentially disrupting another important gene or genomic element. We cannot be certain that off-target editing will not occur in any of our planned or future clinical studies. There is also the potential risk of delayed adverse events following exposure to genome editing therapy due to the potential for persistent biological activity of the genetic material or other components of products used to carry the genetic material.

If any product candidates we develop are associated with serious adverse events, or undesirable side effects, or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe, or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects. Many product candidates that initially showed promise in early stage testing for treating cancer or other diseases have later been found to cause side effects that prevented further clinical development of the product candidates.

If any of the product candidates we may develop or the delivery modes we rely on cause undesirable side effects, it could delay or prevent their regulatory approval, limit the commercial potential, or result in significant negative consequences following any potential marketing approval.

Product candidates we may develop may be associated with off-target editing or other serious adverse events, undesirable side effects, or unexpected characteristics. There also is the potential risk of delayed adverse events following exposure to gene editing therapy due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. In addition to serious adverse events or side effects caused by any product candidate we may develop, the administration process or related procedures also can cause undesirable side effects. If any such events occur, our clinical trials could be suspended or terminated.

If in the future we are unable to demonstrate that such adverse events were caused by factors other than our product candidate, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates we are able to develop for any or all targeted

indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any product candidate we may develop, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to identify and develop product candidates, and may harm our business, financial condition, result of operations, and prospects significantly.

Additionally, if we successfully develop a product candidate and it receives marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy ("REMS") to ensure that the benefits of treatment with such product candidate outweighs the risks for each potential patient, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients, a communication plan to health care practitioners, extensive patient monitoring, or distribution systems and processes that are highly controlled, restrictive, and more costly than what is typical for the industry. Furthermore, if we or others later identify undesirable side effects caused by any product candidate that we to develop, several potentially significant negative consequences could result, including:

· regulatory authorities may suspend or withdraw approvals of such product candidate;

· regulatory authorities may require additional warnings on the label;

· we may be required to change the way a product candidate is administered or conduct additional clinical trials;

· we could be sued and held liable for harm caused to patients; and

· our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of any product candidates we may identify and develop and could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We have not tested any of our proposed delivery modes and product candidates in clinical trials.

Our proposed delivery modes, combined with our product candidates, have never been evaluated in human clinical trials. Moreover, we are not aware of any clinical trials involving CRISPR/Cas9 technology. However, we are aware of at least one group in the United States and at least one group in China that are seeking to initiate clinical studies using CRISPR/Cas9 in the United States and China, respectively. Any product candidates we develop may fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical trials.

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

Any such adverse events may cause us to delay, limit, or terminate planned clinical trials, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

Because we are developing product candidates for the treatment of diseases in which there is little clinical experience using new technologies, there is increased risk that the FDA, the EMA, or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

During the regulatory review process, we will need to identify success criteria and endpoints such that the FDA, the EMA, or other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop. As we are initially seeking to identify and develop product candidates to treat diseases in which there is little clinical experience using new technologies, there is heightened risk that the FDA, the EMA, or other regulatory authorities may not consider the clinical trial endpoints that we propose to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. This may be a particularly significant risk for many of the genetically defined diseases for which we plan to develop product candidates because many of these diseases have small patient populations, and designing and executing a rigorous clinical trial with appropriate statistical power is more difficult than with diseases that have larger patient populations. Further, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the European Union and other countries, such as the CAT, may make similar comments with respect to these endpoints and data. Any product candidates we may develop will be based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. No genome editing product has been approved in the United States or in Europe.

If clinical trials of any product candidates we may identify and develop fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidates.

Before obtaining marketing approval from regulatory authorities for the sale of any product candidates we may identify and develop, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy in humans of any such product candidates. Clinical testing is expensive, difficult to design and implement, can take many years to complete, and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results.

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates.

We or our collaborators may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize any product candidates we may identify and develop, including:

- delays in reaching a consensus with regulators on trial design;
- regulators, institutional review boards (“IRBs”), or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- delays in reaching or failing to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective contract research organizations (“CROs”), and clinical trial sites;

- clinical trials of any product candidates we may develop may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development or research programs;

- difficulty in designing well-controlled clinical trials due to ethical considerations which may render it inappropriate to conduct a trial with a control arm that can be effectively compared to a treatment arm;

- difficulty in designing clinical trials and selecting endpoints for diseases that have not been well-studied and for which the natural history and course of the disease is poorly understood;

- the number of patients required for clinical trials of any product candidates we may develop may be larger than we anticipate; enrollment of suitable participants in these clinical trials, which may be particularly challenging for some of the rare genetically defined diseases we are targeting in our most advanced programs, may be delayed or slower than we anticipate; or subjects may drop out of these clinical trials at a higher rate than we anticipate;

- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;

- regulators, IRBs, or independent ethics committees may require that we or our investigators suspend or terminate clinical research or clinical trials of any product candidates we may develop for various reasons, including noncompliance with regulatory requirements, a finding of undesirable side effects or other unexpected characteristics, or that the participants are being exposed to unacceptable health risks or after an inspection of our clinical trial operations or trial sites;

- the cost of clinical trials of any product candidates we may develop may be greater than we anticipate;

- the supply or quality of any product candidates we may develop or other materials necessary to conduct clinical trials of any product candidates we may develop may be insufficient or inadequate, including as a result of delays in the testing, validation, manufacturing, and delivery of any product candidates we may develop to the clinical sites by us or by third parties with whom we have contracted to perform certain of those functions;

- delays in having subjects complete participation in a trial or return for post-treatment follow-up;

- clinical trial sites dropping out of a trial;

- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;

- occurrence of serious adverse events associated with any product candidates we may develop that are viewed to outweigh their potential benefits;

- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; and

- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

If we or our collaborators are required to conduct additional clinical trials or other testing of any product candidates we may develop beyond those that we currently contemplate, if we or our collaborators are unable to successfully complete clinical trials of any product candidates we may develop or other testing, or if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we or our collaborators may:

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

Product development costs will also increase if we or our collaborators experience delays in testing or marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured, or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize any product candidates we may develop, could allow our competitors to bring products to market before we do, and could impair our ability to successfully commercialize any product candidates we may develop, any of which may harm our business, financial condition, results of operations, and prospects.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We or our collaborators may not be able to initiate or continue clinical trials for any product candidates we identify or develop if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or analogous regulatory authorities outside the United States, or as needed to provide appropriate statistical power for a given trial. Enrollment may be particularly challenging for some of the rare genetically defined diseases we are targeting in our most advanced programs. In addition, if patients are unwilling to participate in our gene editing trials because of negative publicity from adverse events related to the biotechnology, gene therapy, or genome editing fields, competitive clinical trials for similar patient populations, clinical trials in competing products, or for other reasons, the timeline for recruiting patients, conducting studies, and obtaining regulatory approval of any product candidates we may develop may be delayed. Moreover, some of our competitors may have ongoing clinical trials for product candidates that would treat the same indications as any product candidates we may develop, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

Patient enrollment is also affected by other factors, including:

- severity of the disease under investigation;
- size of the patient population and process for identifying subjects;

- design of the trial protocol;
- availability and efficacy of approved medications for the disease under investigation;
- availability of genetic testing for potential patients;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- eligibility and exclusion criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- perceived risks and benefits of genome editing as a therapeutic approach;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

In particular, our most advanced programs are focused on rare genetically defined diseases with limited patient pools from which to draw for enrollment in clinical trials. For example, the global incidence of LCA10 is estimated to be two to three per 100,000 live births worldwide. The eligibility criteria of our clinical trials will further limit the pool of available trial participants. Additionally, the process of finding and diagnosing patients may prove costly.

Our ability to successfully initiate, enroll, and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

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

Enrollment delays in our clinical trials may result in increased development costs for any product candidates we may develop, which would cause the value of our company to decline and limit our ability to obtain additional financing. If we or our collaborators have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit, or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations, and prospects.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial medicines or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable medicines. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing, or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Any such event could have a material adverse effect on our business, financial condition, results of operations, and prospects.

If we are unable to successfully identify patients who are likely to benefit from therapy with any medicines we develop, or experience significant delays in doing so, we may not realize the full commercial potential of any medicines we may develop.

Our success may depend, in part, on our ability to identify patients who are likely to benefit from therapy with any medicines we may develop, which requires those potential patients to have their DNA analyzed for the presence or absence of a particular sequence. For example, although LCA can be diagnosed based on a patient's symptoms and retinal scans, DNA samples are taken from LCA patients in order to test for the presence of the known gene mutations that cause LCA and, where possible, to identify the specific genetically defined disease, such as LCA10. If we, or any third parties that we engage to assist us, are unable to successfully identify such patients, or experience delays in doing so, then:

- our ability to develop any product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;
- any product candidates we develop may not receive marketing approval if safe and effective use of such product candidates depends on an *in vitro* diagnostic; and
- we may not realize the full commercial potential of any product candidates we develop that receive marketing approval if, among other reasons, we are unable to appropriately select patients who are likely to benefit from therapy with our medicines.

As a result, we may be unable to successfully develop and realize the commercial potential of any product candidates we may identify and develop, and our business, financial condition, results of operations, and prospects would be materially adversely effected.

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

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if any product candidates we may develop meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials, and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a REMS. These regulatory authorities

may require precautions or contra-indications with respect to conditions of use, or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of any product candidates we may develop. Any of the foregoing scenarios could materially harm the commercial prospects for any product candidates we may develop and materially adversely affect our business, financial condition, results of operations, and prospects.

Even if any product candidates we may develop receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors, and others in the medical community necessary for commercial success.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors, and others in the medical community. Ethical, social, and legal concerns about genomic medicines generally and genome editing technologies specifically could result in additional regulations restricting or prohibiting our products. Even if any product candidates we may develop receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors, and others in the medical community. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages compared to alternative treatments;
- the limitation to our targeted patient population and limitations or warnings contained in approved labeling by the FDA or other regulatory authority;
- the ability to offer our medicines for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by FDA, the European Commission, or other regulatory agencies;
- public attitudes regarding genomic medicine generally and genome editing technologies specifically;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies, as well as their willingness to accept a therapeutic intervention that involves the editing of the patient's genome;
- product labeling or product insert requirements of the FDA, the EMA, or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- the strength of marketing and distribution support;
- sufficient third-party coverage or reimbursement; and
- the prevalence and severity of any side effects.

If any product candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenues, and we may not become profitable.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any product candidates we may develop, we may not be successful in commercializing those product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing, or distribution of pharmaceutical products. To achieve commercial success for any approved medicine for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing, and commercial support infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates if and when they are approved.

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

Factors that may inhibit our efforts to commercialize our medicines on our own include:

- our inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, customer service, medical affairs, and other support personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future medicines;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary medicines to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

If we enter into arrangements with third parties to perform sales, marketing, commercial support, and distribution services, our product revenues or the profitability of these product revenues to us may be lower than if we were to market and sell any medicines we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our medicines effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.

The development and commercialization of new drug products is highly competitive. Moreover, the genome editing field is characterized by rapidly changing technologies, significant competition, and a strong emphasis on intellectual property. We will face competition with respect to any product candidates that we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have research programs, including LCA10, Duchenne muscular dystrophy, and cystic fibrosis. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches.

Our platform and product focus is the development of therapies using the CRISPR/Cas9 technology. Companies developing the CRISPR/Cas9 technology include Caribou Biosciences, CRISPR Therapeutics, and Intellia Therapeutics. There are additional companies developing therapies using additional genome editing technologies, including transcription activator-like effector nucleases, meganucleases, Mega-TALs, and zinc finger nucleases. These companies include bluebird bio, Cellectis, Poseida Therapeutics, Precision Biosciences, and Sangamo Biosciences. Additional companies developing gene therapy products include Abeona Therapeutics, AGTC Therapeutics, Avalanche Biotechnologies, Dimension Therapeutics, REGENXBIO, Spark Therapeutics, uniQure, and Voyager Therapeutics. In addition to competition from other genome editing therapies or gene therapies, any products we may develop may also face competition from other types of therapies, such as small molecule, antibody, or protein therapies.

Many of our current or potential competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology, and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop or that would render any products that we may develop obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, 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.

Even if we are able to commercialize any product candidates, such products may become subject to unfavorable pricing regulations, third-party reimbursement practices, or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing, and reimbursement for new medicines vary widely from country to country. In the United States, recently enacted legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a medicine before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a medicine in a particular country, but then be subject to price regulations that delay our commercial launch of the medicine, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the medicine in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Our ability to commercialize any medicines successfully also will depend in part on the extent to which reimbursement for these medicines and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any medicine that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved medicines, and coverage may be more limited than the purposes for which the medicine is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that any medicine will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new medicines, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the medicine and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost medicines and may be incorporated into existing payments for other services. Net prices for medicines may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of medicines from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved medicines we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize medicines, and our overall financial condition.

Due to the novel nature of our technology and the potential for any product candidates we may develop to offer therapeutic benefit in a single administration or limited number of administrations, we face uncertainty related to pricing and reimbursement for these product candidates.

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

to market or sell our products. In addition, it may be necessary for us to develop new reimbursement models in order to realize adequate value. Payors may not be able or willing to adopt such new models, and patients may be unable to afford that portion of the cost that such models may require them to bear. If we determine such new models are necessary but we are unsuccessful in developing them, or if such models are not adopted by payors, our business, financial condition, results of operations, and prospects could be adversely affected.

We expect the cost of a single administration of genomic medicine products, such as those we are seeking to develop, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of any such product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of any product candidates we may develop will be paid by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers, and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective, and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical, and cost-effectiveness data. There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize any product candidates we may develop. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

Moreover, the downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any product candidates we may develop will be harmed.

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

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

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any medicines that we may develop.

We face an inherent risk of product liability exposure related to the testing in human clinical trials of any product candidates we may develop and will face an even greater risk if we commercially sell any medicines that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or medicines caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

· decreased demand for any product candidates or medicines that we may develop;

· injury to our reputation and significant negative media attention;

· withdrawal of clinical trial participants;

· significant time and costs to defend the related litigation;

· substantial monetary awards to trial participants or patients;

· loss of revenue; and

· the inability to commercialize any medicines that we may develop.

Although we maintain product liability insurance coverage, it may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage when we begin clinical trials and if we successfully commercialize any medicine. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

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

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state, and local environmental, health, and safety laws, regulations, and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment, and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air, and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research and product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological or hazardous waste insurance coverage, and our commercial general liability and umbrella liability policies (under which we currently have an aggregate of $7.0 million in coverage) specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, we may incur substantial costs in order to comply with current or future environmental, health, and safety laws, regulations, and permitting requirements. These current or future laws, regulations, and permitting requirements may impair our research, development, or production efforts. Failure to comply with these laws, regulations, and permitting requirements also may result in substantial fines, penalties, or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health, and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Genomic medicines are novel, and any product candidates we develop may be complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products, or otherwise harm our business.

Any product candidates we may develop will likely require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as the product candidates we intend to develop generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, or insufficient inventory. If we successfully develop product candidates, we may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, European Commission or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. To date, no cGMP gene therapy manufacturing facility in the United States has received approval from the FDA for the manufacture of an approved genome editing or gene therapy product, and, therefore, the timeframe required for us to obtain such approval is uncertain.

In addition, the FDA, the EMA, and other regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA, or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations, and prospects.

We also may encounter problems hiring and retaining the experienced scientific, quality control, and manufacturing personnel needed to manage our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage. Some of the raw materials that we anticipate will be required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall, or restriction on the use of biologically derived substances in the manufacture of any product candidates we may develop could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations, and prospects.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in third-party

manufacturing process or facilities also could restrict our ability to meet market demand for any products we develop and commercialize.

Risks Related to Our Dependence on Third Parties

We expect to depend on collaborations with third parties for the research, development, and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We anticipate seeking third-party collaborators for the research, development, and commercialization of certain of the product candidates we may develop. For example, in May 2015, we entered into a collaboration with Juno Therapeutics focused on research and development of engineered T cell immunotherapies that utilize or incorporate our genome editing technologies. Our likely collaborators for any other collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, and biotechnology companies. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop, including our collaboration with Juno Therapeutics, pose the following risks to us:

- Collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations. For example, under our collaboration with Juno Therapeutics, development and commercialization plans and strategies for licensed programs will be conducted in accordance with a plan and budget approved by a joint research committee (the "JRC"), comprised of equal numbers of representatives from each of us and Juno Therapeutics.

- Collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities. For example, it is possible for Juno Therapeutics to elect not to submit an IND for a product candidate that we have nominated and the JRC confirmed without triggering a termination of the collaboration arrangement.

- Collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing.

- Collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our medicines or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours.

- Collaborators with marketing and distribution rights to one or more medicines may not commit sufficient resources to the marketing and distribution of such medicine or medicines.

- Collaborators may not properly obtain, maintain, enforce, or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation. For example, Juno Therapeutics has the first right to enforce or defend certain of our intellectual property rights under our collaboration arrangement with respect to certain licensed programs, and although we may have the right to assume the enforcement and defense of such intellectual property rights if Juno Therapeutics does not, our ability to do so may be compromised by Juno Therapeutics' actions.

- Disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our medicines or product candidates or that result in costly litigation or arbitration that diverts management attention and resources.

- We may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control.

- Collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates. For example, Juno Therapeutics can terminate its agreement with us in its entirety upon six months' notice and can terminate the entire agreement with us in connection with a material breach of the agreement by us that remains uncured for a specified period of time.

- Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished, or terminated.

If our collaborations do not result in the successful development and commercialization of products, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval, and commercialization described in this Quarterly Report on Form 10-Q apply to the activities of our collaborators.

We may in the future decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of any product candidates we may develop. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of several factors. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing

products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. For example, during the research program term of our collaboration with Juno Therapeutics, we may not directly or indirectly license, fund, enable, or participate in any research, development, manufacture, or commercialization of engineered T cells with chimeric antigen receptors and T cell receptors in the field of diagnosis, treatment, or prevention of cancer in humans through the use of engineered T cells, excluding the diagnosis, treatment, or prevention of medullary cystic kidney disease.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop product candidates or bring them to market and generate product revenue.

We expect to rely on third parties to conduct our clinical trials and some aspects of our research and preclinical testing, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research, or testing.

We expect to rely on third parties, such as CROs, clinical data management organizations, medical institutions, and clinical investigators, to conduct our clinical trials. We currently rely and expect to continue to rely on third parties to conduct some aspects of our research and preclinical testing. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity, and civil and criminal sanctions.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for any product candidates we may develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our medicines.

We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of any product candidates we may develop or commercialization of our medicines, producing additional losses and depriving us of potential product revenue.

We contract with third parties for the manufacture of materials for our research programs and preclinical studies and expect to continue to do so for clinical trials and for commercialization of any product candidates that we may develop. This reliance on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates, or any medicines that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.

We do not have any manufacturing facilities. We currently rely on third-party manufacturers for the manufacture of our materials for preclinical studies and expect to continue to do so for clinical testing and for commercial supply of any product candidates that we may develop and for which we or our collaborators obtain marketing approval. We do not have a long term supply agreement with any of the third-party manufacturers, and we purchase our required supply on a purchase order basis.

We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the possible breach of the manufacturing agreement by the third party;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and
- reliance on the third party for regulatory compliance, quality assurance, safety, and pharmacovigilance and related reporting.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or medicines, operating restrictions, and criminal prosecutions, any of which could significantly and adversely affect supplies of our medicines and harm our business, financial condition, results of operations, and prospects.

Any medicines that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply for bulk drug substances. If any one of our current contract manufacturer cannot perform as agreed, we may be required to replace that manufacturer. Although we believe that there are several potential alternative manufacturers who could manufacture any product candidates we may develop, we may incur added costs and delays in identifying and qualifying any such replacement.

Our current and anticipated future dependence upon others for the manufacture of any product candidates we may develop or medicines may adversely affect our future profit margins and our ability to commercialize any medicines that receive marketing approval on a timely and competitive basis.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for any products we develop and for our technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize any product candidates we may develop, and our technology may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our CRISPR/Cas9 platform technology and any proprietary product candidates and technology we develop. We seek to protect our proprietary position by in-licensing intellectual property relating to our platform technology and filing patent applications in the United States and abroad related to our technologies and product candidates that are important to our business. If we or our licensors are unable to obtain or maintain patent protection with respect to our CRISPR/Cas9 platform technology and any proprietary products and technology we develop, our business, financial condition, results of operations, and prospects could be materially harmed.

No consistent policy regarding the scope of claims allowable in the field of genome editing, including CRISPR/Cas9 technology, has emerged in the United States. The scope of patent protection outside of the United States is also uncertain. Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions, obtain, maintain, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents. With respect to both in-licensed and owned intellectual property, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors.

The patent prosecution process is expensive, time-consuming, and complex, and we may not be able to file, prosecute, maintain, enforce, or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output in time to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. In addition, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we license or own currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in-license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our platform advances and product candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. For example, we are aware that third parties have suggested the use of the CRISPR technology in conjunction with a protein other than Cas9. Our owned and in-licensed patents may not cover such technology. If our competitors commercialize the CRISPR technology in conjunction with a

protein other than Cas9, our business, financial condition, results of operations, and prospects could be materially adversely affected.

The issuance of a patent is not conclusive as to its inventorship, scope, validity, or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. We may be subject to a third party preissuance submission of prior art to the United States Patent and Trademark Office (the "USPTO"), or become involved in opposition, derivation, revocation, re-examination, post-grant and *inter partes* review, or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge priority of invention or other features of patentability. Such challenges may result in loss of patent rights, loss of exclusivity, or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. As discussed below, some of our in-licensed patents are subject to interference, opposition, and *ex parte* re-examination proceedings and therefore subject to these risks.

In addition, given the amount of time required for the development, testing, and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Moreover, some of our owned and in-licensed patents and patent applications are, and may in the future be, co-owned with third parties. If we are unable to obtain an exclusive license to any such third party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Furthermore, our owned and in-licensed patents may be subject to a reservation of rights by one or more third parties. For example, the research resulting in certain of our owned and in-licensed patent rights and technology was funded in part by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights could harm our competitive position, business, financial condition, results of operations, and prospects.

Our rights to develop and commercialize our technology and product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.

We are heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our genome editing technology, including our CRISPR/Cas9 technology, and product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from

developing and commercializing competitive products in territories included in all of our licenses. For example, pursuant to our license agreement (the "Broad-Harvard License Agreement") with The Broad Institute, Inc. ("Broad") and the President and Fellows of Harvard College ("Harvard"), under certain circumstances, Broad and Harvard may grant a license to the patents that are the subject of our license agreement to a third party. Such third party would have full rights to the patent rights that are the subject of our Broad-Harvard License Agreement, which could impact our competitive position and enable a third party to commercialize products similar to our future product candidates and technology.

In addition, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. For example, pursuant to each of our intellectual property licenses with Broad and Harvard, The General Hospital Corporation, d/b/a Massachusetts General Hospital and Duke University, our licensors retain control of preparation, filing, prosecution, and maintenance, and, in certain circumstances, enforcement and defense of their patents and patent applications. Therefore, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our licensors fail to prosecute, maintain, enforce, and defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any of our products that are subject of such licensed rights could be adversely affected. Additionally, given that we are required to reimburse our licensors for all of their expenses related the prosecution, maintenance, enforcement and defense of patents and patent applications that we in-license from them, the ongoing nature of the interference and opposition proceedings involving the patents licensed to us under the Broad-Harvard License Agreement and our obligation to make such reimbursements are not subject to any limitations, we anticipate that our obligation to reimburse our licensors for expenses related to these matters will continue to be substantial.

Our licensors may have relied on third party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents we in-licensed. For example, the Rockefeller University ("Rockefeller") is a joint applicant on certain patent applications (including a continuation of one of these applications) that we have in-licensed from Broad, acting on behalf of itself, Massachusetts Institute of Technology ("MIT") and Harvard. Broad does not and does not purport to grant any rights in Rockefeller's interest in these patent applications under our agreement. As a result, Broad may not be the sole and exclusive owner of any patents that issue from these patent applications. If other third parties have ownership rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties (potentially including our competitors) to receive licenses to a portion of the intellectual property that is subject to our existing licenses. Any of these events could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Some of our in-licensed patents are subject to priority disputes. In addition, our owned and in-licensed patents and other intellectual property may be subject to further priority disputes or to inventorship disputes and similar proceedings including validity disputes. If we or our licensors are unsuccessful in any of these proceedings, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or to cease the development, manufacture, and commercialization of one or more of the product candidates we may develop, which could have a material adverse impact on our business.

On January 11, 2016, the Patent Trial and Appeal Board of the USPTO (the "PTAB") declared an interference between a pending U.S. patent application (U.S. Serial No. 13/842,859) that is owned by the University of California,

the University of Vienna, and Emmanuelle Charpentier and 12 U.S. patents (U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; and 8,999,641) that are co-owned by Broad and MIT, and in some cases Harvard, and in-licensed by us. On March 17, 2016, the PTAB re-declared the interference to add a pending U.S. patent application (U.S. Serial No. 14/704,551) that is co-owned by Broad, MIT, and Harvard, and in-licensed by us. An interference is a proceeding within the USPTO to determine priority of invention of the subject matter of patent claims filed by different parties. This proceeding is only potentially available for patent applications filed in the United States on or before March 15, 2013 and related continuing patent applications.

In the declared interference, the University of California, acting on behalf of itself and the University of Vienna, and Emmanuelle Charpentier have been designated as the senior party and Broad has been designated as the junior party. In an interference proceeding, the junior party has the burden of proof and presents its priority evidence first. The declaration of interference defines the invention that is subject to the declaration of interference, also referred to as "the count," as relating to a method that involves contacting a target DNA in a eukaryotic cell with certain defined CRISPR/Cas9 components for the purpose of cleaving or editing a target DNA molecule or modulating transcription of at least one gene encoded thereon. All of the claims in the pending U.S. patent application that is owned by the University of California, the University of Vienna, and Emmanuelle Charpentier and all of the claims in the 12 U.S. patents and one pending U.S. patent application that are co-owned by Broad and MIT, and in some cases Harvard, and in-licensed by us are currently implicated in the interference. Prior to the declaration of interference, the University of California, acting on behalf of itself and the University of Vienna, and Emmanuelle Charpentier filed a "Suggestion of Interference" in the USPTO on April 13, 2015, which requested that an interference be declared between certain claims in this same pending U.S. patent application (U.S. Serial No. 13/842,859) and certain claims in 10 U.S. patents, which we have in-licensed from Broad, acting on behalf of itself, MIT, and Harvard. A Supplemental Suggestion of Interference was filed by the University of California and Emmanuelle Charpentier on November 5, 2015, which requested that an interference be declared between certain claims in their same pending U.S. patent application (U.S. Serial No. 13/842,859) and certain claims in two additional U.S. patents and five pending U.S. applications (including U.S. Serial No. 14/704,551 which has now been added to the interference), which we have in-licensed from Broad, acting on behalf of itself, MIT, and Harvard. The 12 U.S. patents referred to in the Suggestion of Interference and Supplemental Suggestion of Interference are the same as those included in the declaration of interference. The Suggestion of Interference and Supplemental Suggestion of Interference assert that the inventors from the University of California and the University of Vienna, and Emmanuelle Charpentier made certain inventions before the inventors from Broad and MIT and, in certain cases, Harvard, which will be evaluated by the PTAB in the interference discussed above. The University of California, the University of Vienna, and Emmanuelle Charpentier are listed as applicants on U.S. Serial No. 13/842,859. The University of California derives rights in U.S. Serial No. 13/842,859 from an assignment by Dr. Jennifer Doudna and certain other inventors listed on such application. Caribou Biosciences has reported that it has an exclusive license to patent rights from the University of California and the University of Vienna. Intellia Therapeutics has reported that it has an exclusive license to such rights from Caribou Biosciences in certain fields. CRISPR Therapeutics has reported that it has an exclusive license to patent rights from Emmanuelle Charpentier. Further, Dr. Doudna was a founder of our company and entered into a consulting agreement with us at the time of our founding. However, Dr. Doudna gave notice of termination of that agreement in May 2014 after less than seven months of service, and she has had no further engagement in our business since that time. Dr. Doudna is also a founder of Caribou Biosciences and has been publicly identified as an advisor to Intellia Therapeutics, each of which is one of our competitors.

As a result of the declaration of interference, an adversarial proceeding in the USPTO before the PTAB has been initiated. An interference is declared to ultimately determine priority, specifically which party was first to invent the commonly claimed invention. An interference is typically divided into two phases. The first phase is typically referred to as the motions or preliminary motions phase while the second is referred to as the priority phase. In the first phase, each party may raise issues including but not limited to those relating to the patentability of a party's claims based on prior art, written description, and enablement. A party also may seek an earlier priority benefit or may challenge whether the declaration of interference was proper in the first place. Priority, or a determination of who first invented the commonly claimed invention, is determined in the second phase of an interference. Although we cannot predict with any certainty how long each phase will actually take, each phase may take approximately a year or longer before a decision is made by the PTAB. It is possible for motions filed in the preliminary motions phase to be dispositive of the interference proceeding, such that the second priority phase is not reached. It is also possible that other third parties may

seek to become a party to this interference or a future interference or that the University of California and Emmanuelle Charpentier or other third parties may file a separate Suggestion of Interference against the Broad patents subject to the interference or other U.S. patents and patent applications that we own or in-license.

Separately, ToolGen Inc. ("ToolGen") filed Suggestions of Interference in the USPTO on April 13, 2015, which became publicly available on November 12, 2015 and December 3, 2015, suggesting that they believe some of the claims in pending U.S. applications owned by ToolGen (U.S. Serial No. 14/685,568 and U.S. Serial No. 14/685,510, respectively) interfere with certain claims in five U.S. patents, which we have in-licensed from Broad, acting on behalf of itself, MIT, and Harvard. These five U.S. patents are among the 12 U.S. patents with respect to which the PTAB has declared an interference. The Suggestions of Interference that were filed by ToolGen are still pending and it is uncertain when and in what manner the USPTO will act on them.

We or our licensors are subject to validity disputes in the USPTO and in the future may become a party to additional validity disputes in the United States or other jurisdictions. A request for *ex parte* re-examination was filed with the USPTO on February 16, 2016 against one of the patents with respect to which the PTAB has declared an interference (U.S. Patent No. 8,771,945). This patent is also one of the patents that ToolGen has included in its Suggestions of Interference. *Ex parte* re-examination is a procedure through which a third party can anonymously request the USPTO to re-examine a granted patent because the third party believes the granted patent may not be patentable over prior art in the form of a printed publication or another patent. Before the USPTO will re-examine a granted patent, the third party requestor must establish that the submitted prior art establishes a substantial and new question of patentability. If the USPTO determines there is a substantial and new question of patentability, it grants the re-examination request and re-examines the patent after giving the patent owner the option of filing an initial statement. The request for *ex parte* re-examination of U.S. Patent No. 8,771,945 was granted on May 9, 2016 thereby initiating a re-examination procedure between the USPTO and Broad, acting on behalf of itself and MIT. The third party requestor does not participate in the re-examination procedure after filing the request except that it has the option of responding if the patent owner chooses to file an initial statement. On May 12, 2016, the PTAB suspended the re-examination of U.S. Patent No. 8,771,945 noting that it has jurisdiction over any file that involves a patent involved in the interference. It is uncertain when the PTAB will lift the suspension.

The 12 in-licensed U.S. patents and one in-licensed U.S. patent application that are the subject of the interference with the pending U.S. patent application (U.S. Serial No. 13/842,859) that is owned by the University of California, the University of Vienna, and Emmanuelle Charpentier (which includes the five in-licensed U.S. patents that are the subject of the Suggestions of Interference filed by ToolGen and the one in-licensed U.S. patent that is the subject of the re-examination) relate generally to the CRISPR/Cas9 system and its use in eukaryotic cells. The claims of the 12 in-licensed U.S. patents and one in-licensed U.S. patent application vary in scope and coverage and include claims that are directed to CRISPR/Cas9 systems that employ viral vectors for delivery, single guide RNAs, modified guide RNAs, *S. aureus* Cas9, or a Cas9 nickase and are relevant to our genome editing platform technology. The loss of one or more of these in-licensed patents could have a material adverse effect on the conduct of our business.

In addition, we or our licensors may be subject to claims that former employees, collaborators, or other third parties have an interest in our owned or in-licensed patents or other intellectual property as an inventor or co-inventor. For example, we have determined that certain of the claims of one of our pending U.S. non-provisional patent applications, and its corresponding pending PCT application, cover subject matter invented jointly by us and other third parties, which will result in certain third parties holding co-ownership rights in such applications. If we are unable to obtain an exclusive license to any such third party co-owners' interest in such patent applications, such co-owners may be able to license their rights to other third parties, including our competitors. In addition, we may need the cooperation of any such co-owners to enforce any patents that issue from such patent applications against third parties, and such cooperation may not be provided to us. We are also aware of one third party, Rockefeller, that has independently filed a U.S. patent application (U.S. Serial No. 14/324,960) as a continuation of a U.S. patent application that we have in-licensed from Broad, acting on behalf of itself and MIT (U.S. Serial No. 14/183,429, which has since issued as U.S. Patent No. 8,771,945). In contrast to a Suggestion of Interference, a U.S. continuation patent application does not seek to challenge the priority date of an existing patent, rather it is a new filing of an existing U.S. patent application, which contains the same priority date as the existing application. However, it may provoke the declaration of an interference. In that regard, the U.S. continuation patent application filed by Rockefeller lists one of its employees as a co-inventor

alongside Dr. Feng Zhang, who is an employee of Broad in addition to being one of our founders. The U.S. continuation patent application was filed by Rockefeller with copies of claims from one U.S. patent and one U.S. patent application, which we have in-licensed from Broad, acting on behalf of itself and MIT (U.S. Patent No. 8,697,359 and U.S. Serial No. 14/183,429, which has since issued as U.S. Patent No. 8,771,945). The U.S. continuation patent application filed by Rockefeller may provoke the declaration of an interference by the USPTO with these or other patents that we have in-licensed from Broad, acting on behalf of itself, MIT, and Harvard. The U.S. continuation application filed by Rockefeller may also prompt a derivation proceeding in the USPTO or litigation in court regarding such continuation patent application. In addition, if the USPTO were to grant a patent based on this U.S. continuation patent application including the Rockefeller employee as an inventor, then Rockefeller could license its rights to such patent to one of our competitors or to another third party such that they may have freedom-to-operate under such patent and may commercialize similar or identical products and technology to us. We may also need the cooperation of Rockefeller to enforce such patent against third parties, and such cooperation may not be provided to us. Any of the foregoing could have a material adverse effect on the conduct of our business.

We or our licensors are subject to and may in the future become a party to similar proceedings or priority disputes in Europe or other foreign jurisdictions. The European Patent Office Opposition Division has initiated opposition proceedings in the European Patent Office (the "EPO") against three European patents that we have in-licensed from Broad, acting on behalf of itself, MIT and Harvard (European Patent Nos. EP 2,771,468 B1, EP 2,784,162 B1 and EP 2,896,697 B1) and one European patent that we have in-licensed from Broad, acting on behalf of itself and MIT (European Patent No. EP 2,764,103 B1). The EPO opposition proceedings may involve issues including, but not limited to, procedural formalities related to filing the European patent application, priority, and the patentability of the involved claims. One or more of the third parties that have filed oppositions against European Patent Nos. EP 2,771,468 B1, EP 2,784,162 B1, EP 2,764,103 B1, and/or EP 2,896,697 B1 or other third parties may file future oppositions against other European patents that we in-license or own. For example, we are aware that oppositions have been filed against three other European patents that we in-license from Broad, acting on behalf of itself, MIT and Harvard (European Patent Nos. EP 2,898,075 B1, EP 2,931,898 B1 and EP 2,921,557 B1). The deadlines for filing oppositions against these European patents are December 9, 2016 for the first two European patents and April 13, 2017 for the third European patent. There may be other oppositions against these European patents that have not yet been filed or that have not yet been made available to the public. The loss of priority for, or the loss of, these European patents could have a material adverse effect on the conduct of our business.

If we or our licensors are unsuccessful in any interference proceedings or other priority or validity disputes (including any patent oppositions or re-examinations) to which we or they are subject, we may lose valuable intellectual property rights through the loss of one or more patents owned or licensed or our owned or licensed patent claims may be narrowed, invalidated, or held unenforceable. In addition, if we or our licensors are unsuccessful in any inventorship disputes to which we or they are subject, we may lose valuable intellectual property rights, such as exclusive ownership of, or the exclusive right to use, our owned or in-licensed patents. If we or our licensors are unsuccessful in any interference proceeding or other priority or inventorship dispute, we may be required to obtain and maintain licenses from third parties, including parties involved in any such interference proceedings or other priority or inventorship disputes. Such licenses may not be available on commercially reasonable terms or at all, or may be non-exclusive. If we are unable to obtain and maintain such licenses, we may need to cease the development, manufacture, and commercialization of one or more of the product candidates we may develop. The loss of exclusivity or the narrowing of our owned and licensed patent claims could limit our ability to stop others from using or commercializing similar or identical technology and products. Any of the foregoing could result in a material adverse effect on our business, financial condition, results of operations, or prospects. Even if we are successful in an interference proceeding or other similar priority or inventorship disputes, it could result in substantial costs and be a distraction to management and other employees.

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

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of 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 intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary 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, could put 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 and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by government 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 government fees on patents and applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned or licensed patents and applications. In certain circumstances, we rely on our licensing partners to pay these fees due to U.S. and non-U.S. patent agencies. The USPTO and various non-U.S. government agencies require compliance with several procedural, documentary, fee payment, and other similar provisions during the patent application process. We are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In some cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in a partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market with similar or identical products or technology, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

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 have entered into license agreements with third parties and may need to obtain additional licenses from our existing licensors and others to advance our research or allow commercialization of product candidates we may develop. It is possible that we may be unable to obtain any additional 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 redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations,

and prospects significantly. We cannot provide any assurances that third party patents do not exist which might be enforced against our current technology, including CRISPR/Cas9, manufacturing methods, product candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

In each of our license agreements, and we expect in our future agreements, we are responsible for bringing any actions against any third party for infringing on the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products. 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 inventorship and 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.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

We may not be successful in obtaining necessary rights to any product candidates we may develop through acquisitions and in-licenses.

We currently have rights to intellectual property, through licenses from third parties, to identify and develop product candidates. Many pharmaceutical companies, biotechnology companies, and academic institutions are competing with us in the field of genome editing technology and filing patent applications potentially relevant to our business. For example, we are aware of several third party patent applications that, if issued, may be construed to cover our CRISPR/Cas9 technology and product candidates. In order to avoid infringing these third party patents, we may find it necessary or prudent to obtain licenses from such third party intellectual property holders. We may also require licenses from third parties for certain non-CRISPR/Cas9 technologies including certain delivery methods that we are evaluating for use with product candidates we may develop. In addition, with respect to any patents we co-own with third parties, we may require licenses to such co-owners' interest to such patents. However, we may be unable to secure such licenses or otherwise acquire or in-license any compositions, methods of use, processes, or other intellectual property rights from third parties that we identify as necessary for product candidates we may develop and CRISPR/Cas9 technology. The licensing or acquisition of third party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. 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 or at all. For example, certain delivery modes, including certain adeno-associated virus vectors and lipid nanoparticle technologies, we are evaluating for use in our LCA10 program or with other product candidates we may develop are covered by patents held by third parties. If we are unable to successfully obtain rights to required third party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act (the "America Invents Act"), enacted in September 2011, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. 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. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Issued patents covering our technology and product candidates could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.

If we or one of our licensors initiated legal proceedings against a third party to enforce a patent covering a product candidate we may develop or our technology, including CRISPR/Cas9, the defendant could counterclaim that such patent is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties have raised challenges to the validity of certain of our in-licensed patent claims and may in the future 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, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). For example, an interference has been declared against 12 of our in-licensed U.S. patents and one pending U.S. patent application, one of these U.S. patents is subject to a re-examination, opposition proceedings have been initiated against four of our in-licensed European patents, oppositions have been filed against another three of our in-licensed European patents, and additional interference, re-examination and opposition proceedings may be initiated in the future. For more information regarding these

proceedings, see “Part II, Item 1. Legal Proceedings” of our Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, which was filed with the SEC on August 10, 2016. Such proceedings could result in the revocation of, cancellation of, or amendment to our patents in such a way that they no longer cover our technology or platform, or any product candidates that we may develop. 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 or our licensing partners and the patent examiner were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology or platform, or any product candidates that we may develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations, and prospects.

The intellectual property landscape around genome editing technology, including CRISPR/Cas9, is highly dynamic, and third parties may initiate legal proceedings alleging that we are infringing, misappropriating, or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

The field of genome editing, especially in the area of CRISPR/Cas9 technology, is still in its infancy, and no such products have reached the market. Due to the intense research and development that is taking place by several companies, including us and our competitors, in this field, the intellectual property landscape is in flux, and it may remain uncertain for the coming years. There may be significant intellectual property related litigation and proceedings relating to our owned and in-licensed, and other third party, intellectual property and proprietary rights in the future.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market, and sell any product candidates that we may develop and use our proprietary technologies without infringing, misappropriating, or otherwise violating the intellectual property and proprietary rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive litigation regarding patents and other intellectual property rights. We are subject to and may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our technology and any product candidates we may develop, including interference proceedings, post-grant review, *inter partes* review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions such as oppositions before the EPO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. We are aware of certain third party patent applications in this landscape that may, if issued as patents, be asserted to encompass our CRISPR/Cas9 technology. In particular, we are aware of several separate families of U.S. patent applications and foreign counterparts which relate to CRISPR/Cas9 technology, where the earliest priority dates of each family pre-date the priority dates of our in-licensed patents and patent applications, including PCT Publication No. WO 2013/176772 (and its related U.S. and foreign patent applications) filed by the University of California, the University of Vienna (both of which are reported to have exclusively licensed their rights to Caribou Biosciences, which is reported to have exclusively licensed certain rights to Intellia Therapeutics), and Emmanuelle Charpentier (who is reported to have exclusively licensed her rights to CRISPR Therapeutics), and WO 2014/065596 (and its related U.S. patent applications and foreign patent and patent applications) filed by ToolGen. Each of these patent families are owned by a different third party and contain claims that may be construed to cover components and uses of CRISPR/Cas9 technology. If these third-party patent applications are issued as patents and we are not able to obtain or maintain a license on commercially reasonable terms to any third-party patents that cover our product candidates or activities, such third parties could potentially assert infringement claims against us, which could have a material adverse effect on the conduct of our business. We are also aware of third-party U.S. patents (U.S. Patent No. 8,921,332 and U.S. Patent No. 9,458,439), which are reported to have been exclusively licensed to Cellectis and contain claims related to methods for inducing double strand breaks in chromosomal DNA using a chimeric restriction endonuclease. In addition, we are aware of a U.S. patent and a related U.S. continuation patent application (U.S. Patent No. 9,200,266 and U.S. Serial No. 14/925,386) that is assigned to Sangamo Biosciences, Inc. and contains claims to a chimeric nuclease that induces a site-specific single-stranded break in a double-stranded DNA. Although we believe that we do not infringe a valid claim of such third party patents, such third parties may assert infringement claims or claim infringement against us, and if we are found to infringe such third party patents, we may be required to pay damages, cease commercialization of the infringing technology, or obtain a license from such third parties, which may not be available on commercially reasonable terms or at all.

Even if we believe third-party intellectual property claims are without merit, there is no assurance that a court would find in our favor on questions of infringement, validity, enforceability, or priority. A court of competent jurisdiction could hold that these third party patents are valid, enforceable, and infringed, which could materially and adversely affect our ability to commercialize any product candidates we may develop and any other product candidates or technologies covered by the asserted third party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's intellectual property rights, and we are unsuccessful in demonstrating that such patents are invalid or unenforceable, we could be required to obtain a license from such third party to continue developing, manufacturing, and marketing any product candidates we may develop and our technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We also could be forced, including by court order, to cease developing, manufacturing, and commercializing the infringing technology or product candidates. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar material adverse effect on our business, financial condition, results of operations, and prospects.

We may be subject to claims that our employees, consultants, or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants, and advisors are currently or 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 advisors 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 these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. 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. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We may become involved in lawsuits to protect or enforce our patents and other intellectual property rights, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensing partners, or we may be required to defend against claims of infringement. In addition, our patents or the patents of our licensing partners also are, and may in the future become, involved in inventorship or priority disputes. To counter or defend against such claims can be expensive and time consuming. In an infringement proceeding, a court may decide that a patent owned or in-licensed by us is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our owned and in-licensed patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our owned or in-licensed patents at risk of being invalidated or interpreted narrowly. 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.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing, or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and product candidates, we also rely on trade secrets and confidentiality agreements to protect our unpatented know-how, technology, and other proprietary information and to maintain our competitive position. With respect to our technology platform, we consider trade secrets and know-how to be one of our primary sources of intellectual property. Trade secrets and know-how can be difficult to protect. In particular, we anticipate that with respect to our technology platform, these trade secrets and know-how will over time be disseminated within the industry through independent development, the publication of journal articles describing the methodology, and the movement of personnel from academic to industry scientific positions.

We seek to protect these trade secrets and other proprietary technology, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our competitive position would be materially and adversely harmed.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984 (the “Hatch-Waxman Amendments”). The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent

protection afforded could be less than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to any product candidates we may develop or utilize similar gene therapy technology but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending licensed patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming, and uncertain and may prevent us from obtaining approvals for the commercialization of any product candidates we may develop. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, product candidates we may develop, and our ability to generate revenue will be materially impaired.

Any product candidates we may develop and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage,

approval, advertising, promotion, sale, and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the biologic product candidate's safety, purity, and potency. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we develop may not be effective, may be only moderately effective, or may prove to have undesirable or unintended side effects, toxicities, or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity, and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical, or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit, or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved medicine not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the European Union and many other foreign jurisdictions, we or our third-party collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Even if we, or any collaborators we may have, obtain marketing approvals for any product candidates we develop, the terms of approvals and ongoing regulation of our products could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising, and promotional activities for such medicine, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include

submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine.

Accordingly, assuming we, or any collaborators we may have, receive marketing approval for one or more product candidates we develop, we, and such collaborators, and our and their contract manufacturers will continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, and quality control. If we and such collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition, and prospects.

Any product candidate for which we obtain marketing approval could be subject to restrictions or withdrawal from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our medicines, when and if any of them are approved.

The FDA and other regulatory agencies closely regulate the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other regulatory agencies impose stringent restrictions on manufacturers' communications regarding off-label use, and if we do not market our medicines for their approved indications, we may be subject to enforcement action for off-label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice. Violation of the Federal Food, Product, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown problems with our medicines, manufacturers, or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such medicines, manufacturers, or manufacturing processes;
- restrictions on the labeling or marketing of a medicine;
- restrictions on the distribution or use of a medicine;
- requirements to conduct post-marketing clinical trials;
- receipt of warning or untitled letters;
- withdrawal of the medicines from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of medicines;
- fines, restitution, or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- suspension of any ongoing clinical trials;

- refusal to permit the import or export of our medicines;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

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 product candidates we may develop and adversely affect our business, financial condition, results of operations, and prospects.

Our relationships with healthcare providers, physicians, and third-party payors will be subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, and diminished profits and future earnings.

Healthcare providers, physicians, and third-party payors play a primary role in the recommendation and prescription of any product candidates that we may develop for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell, and distribute our medicines for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving, or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order, or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment or approval from Medicare, Medicaid, or other government payors that are false or fraudulent or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at $5,500 to $11,000 per false claim;
- the federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”), as further amended by the Health Information Technology for Economic and Clinical Health Act (“HITECH”), which imposes certain requirements, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses, and health care providers;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing, or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items, or services;
- the federal transparency requirements under the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics, and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value to physicians and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and

- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

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 business, financial condition, results of operations, and prospects.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order, or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publically 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.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations, or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal, and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil, or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Future legislation, and any changes to existing legislation, may increase the difficulty and cost for us and any collaborators we may have to obtain marketing approval of and commercialize any product candidates we may develop and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been and continue to be a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of any product candidates that we may develop, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or our future collaborators, may receive for any approved products.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (the "Medicare Modernization Act") changed the way Medicare covers and pays for pharmaceutical products. The legislation

expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payors.

The Patient Protection and Affordable Care Act enacted in March 2010 and subsequently amended by the Health Care and Education Affordability Reconciliation Act (collectively, the "PPACA") contains several provisions of potential importance to any product candidates we may develop, including the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription products and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Product Rebate Program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for non-compliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report product samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any product candidates we may develop for which marketing approval is obtained.

We expect that the PPACA, as well as other healthcare reform measures that 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. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue from sales of products, attain profitability, or commercialize any product candidates we may develop.

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

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

Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain, and motivate qualified personnel.

We are highly dependent on Katrine S. Bosley, our Chief Executive Officer, as well as the other principal members of our management and scientific teams. Ms. Bosley is employed “at will,” meaning we or she may terminate the employment relationship at any time. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development, and commercialization objectives.

Recruiting and retaining qualified scientific, clinical, manufacturing, and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors, including our scientific co-founders, may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. The inability to recruit, or loss of services of certain executives, key employees, consultants, or advisors, may impede the progress of our research, development, and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations, and prospects.

We expect to expand our development, regulatory, and future sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational, and financial systems, expand our facilities, and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expected expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected physical expansion of our operations may lead to significant costs and may divert our

management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to Our Common Stock

An active trading market for our common stock may not be sustained

Our shares of common stock began trading on The NASDAQ Global Select Market on February 3, 2016. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares.

The market price of our common stock may be volatile, which could result in substantial losses for our stockholders.

Our stock price is, and is likely to continue to be, volatile. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the prices at which they purchased their shares. Some of the factors that may cause the market price of our common stock to fluctuate include:

- the success of existing or new competitive products or technologies;
- the timing and results of preclinical studies for our LCA10 program and any product candidates that we may develop;
- commencement or termination of collaborations for our product development and research programs;
- failure or discontinuation of any of our product development and research programs;
- results of preclinical studies, clinical trials, or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- developments or changing views regarding the use of genomic medicines, including those that involve genome editing;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents, or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our research programs, clinical development programs, or product candidates that we may develop;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines, or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders, or other stockholders;
- expiration of market stand-off or lock-up agreements;

· variations in our financial results or those of companies that are perceived to be similar to us;

· changes in estimates or recommendations by securities analysts, if any, that cover our stock;

· changes in the structure of healthcare payment systems;

· market conditions in the pharmaceutical and biotechnology sectors;

· general economic, industry, and market conditions; and

· the other factors described in this "Risk Factors" section.

In recent years, the stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. Following periods of such volatility in the market price of a company's securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future. Securities litigation could result in substantial costs and divert management's attention and resources from our business.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock and trading volume could decline.

The trading market for our common stock depends in part on the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

A significant portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock.

All lock-up agreements entered into in connection with our initial public offering expired on July 31, 2016. Our outstanding shares of common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended (the "Securities Act"), or to the extent that such shares have already been registered under the Securities Act and are held by non-affiliates of ours.

Moreover, holders of a substantial number of shares of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also have registered substantially all shares of common stock that we may issue under our equity compensation plans or that are issuable upon exercise of outstanding options. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

Our executive officers, directors, and principal stockholders, if they choose to act together, have the ability to control all matters submitted to stockholders for approval.

As of November 1, 2016, our executive officers and directors, combined with our stockholders who owned more than 5% of our outstanding common stock, and their affiliates, in the aggregate, beneficially owned shares

representing a majority of our outstanding common stock. As a result, these stockholders, if they were to act together, would be able to influence our management and affairs and all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions. This concentration of ownership may have the effect of delaying or preventing a change in control of our company and might affect the market price of our common stock.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"), and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 ("SOX Section 404"), not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. As a result, the information we provide stockholders will be different than the information that is available with respect to other public companies. We expect to continue to take advantage of some of the reporting exemptions available to emerging growth companies. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock, and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards, and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

As compared to previous years, we will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an "emerging growth company," we will incur significant legal, accounting, and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Select Market, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional accounting, finance, and other personnel in connection with our becoming, and our efforts to comply with the requirements of being, a public company, and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. We are currently evaluating these rules and regulations and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to SOX Section 404, we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the SEC after we

become a public company. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with SOX Section 404 within the prescribed period, we are engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented, and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We have broad discretion in the use of our cash reserves and may not use them effectively.

Our management has broad discretion to use our cash reserves and could use our cash reserves in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline, and delay the development of our product candidates. Pending their use, we may invest our cash reserves in a manner that does not produce income or that loses value.

We do not expect to pay any dividends for the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investments.

We have never declared or paid cash dividends on our common stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

Provisions in our certificate of incorporation and bylaws or Delaware law might discourage, delay, or prevent a change in control of our company or changes in our management and, therefore, depress the trading price of our common stock.

Provisions in our certificate of incorporation and bylaws or Delaware law may discourage, delay, or prevent a merger, acquisition, or other change in control that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares of our common stock. These provisions may also prevent or frustrate attempts by our stockholders to replace or remove our management. These provisions include:

- limitations on the removal of directors;
- a classified board of directors so that not all members of our board of directors are elected at one time;
- advance notice requirements for stockholder proposals and nominations;
- the inability of stockholders to act by written consent or to call special meetings;
- the requirement that at least 75% of the votes cast by all our stockholders approve the amendment or repeal of certain provisions of our bylaws or certificate of incorporation;
- the ability of our board of directors to make, alter, or repeal our bylaws; and
- the ability of our board of directors to designate the terms of and issue new series of preferred stock without stockholder approval, which could be used to institute a rights plan, or a poison pill, that would work to dilute the stock ownership of a potential hostile acquirer, likely preventing acquisitions that have not been approved by our board of directors.

In addition, Section 203 of the General Corporation Law of the State of Delaware prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person which together with its affiliates owns, or within the last three years has owned, 15% of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner.

The existence of the foregoing provisions could also deter potential acquirers of our company, thereby reducing the likelihood that our stockholders could receive a premium for their shares of common stock in an acquisition.

Our certificate of incorporation designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against the company and our directors and officers.

Our certificate of incorporation provides that, unless our board of directors otherwise determines, the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors or officers to our company or our stockholders, any action asserting a claim against us or any of our directors or officers arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or bylaws, or any action asserting a claim against us or any of our directors or officers governed by the internal affairs doctrine. This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors or officers, which may discourage such lawsuits against us and our directors and officers.

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

Use of Proceeds from Registered Securities

On February 8, 2016, we closed our initial public offering of common stock under a registration statement on Form S-1 (File No. 333-208856) that was declared effective by the Securities and Exchange Commission (the "SEC") on February 2, 2016.

We received aggregate net proceeds from the offering of $97.5 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. None of the underwriting discounts and commissions or other offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10% or more of our common stock or to any affiliates of ours.

As of September 30, 2016, we had used approximately $10.1 million of the net offering proceeds, primarily to fund preclinical studies for our LCA10 program, continued expansion of our platform technology, and preclinical studies of our research programs in addition to LCA10 and engineered T cells, as well as for working capital and general corporate purposes. We have invested the remaining net proceeds from the offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) under the Securities Act of 1933, as amended.

Item 6. Exhibits

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

SIGNATURES

Pursuant to the requirements of 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.

EDITAS MEDICINE, INC.

Dated: November 9, 2016

By: /s/ Andrew A. F. Hack
Andrew A. F. Hack M.D., Ph.D.
Chief Financial Officer
(Principal Financial Officer)

Exhibit Number	Description of Exhibit
10.1†	Exclusive Patent License Agreement, dated August 2, 2016, between the Registrant and The General Hospital Corporation, d/b/a Massachusetts General Hospital
10.2	Sublease Amendment No.1, dated August 31, 2016, between the Registrant and Alnylam Pharmaceuticals, Inc. (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K (File No. 001-37687) filed with the Securities and Exchange Commission on September 2, 2016)
31.1	Rule 13a-14(a) Certification of Principal Executive Officer
31.2	Rule 13a-14(a) Certification of Principal Financial Officer
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. §1350
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

† Confidential treatment requested as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission.

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

THE GENERAL HOSPITAL CORPORATION

EXCLUSIVE PATENT LICENSE AGREEMENT

MGH Agreement No: A224596
MGH Case Nos: [**]

This License Agreement ("Agreement") is made as of the 2nd day of August, 2016 ("Effective Date"), by and between Editas Medicine, Inc., a Delaware corporation, with its principal place of business located at 300 Third Street, Cambridge, MA 02142 ("Company"), and The General Hospital Corporation, d/b/a Massachusetts General Hospital, a not-for-profit Massachusetts corporation, with a principal place of business at 55 Fruit Street, Boston, Massachusetts 02114 ("Hospital"), each referred to herein individually as a "Party" and collectively as the "Parties."

RECITALS

Hospital, as a center for patient care, research and education, is the owner of certain Patent Rights and Technological Information (defined below) and desires to grant a license of those Patent Rights and Technological Information to Company in order to benefit the public by disseminating the results of its research via the commercial development, manufacture, distribution and use of Product and Process (defined below).

Company has the capability to commercially develop, manufacture, distribute and use Product and Process for public use and benefit and desires to license such Patent Rights and Technological Information.

For good and valuable consideration, the sufficiency of which is hereby acknowledged, the Parties hereby agree as follows:

ARTICLE 1. CERTAIN DEFINITIONS

As used in Agreement, the following terms shall have the following meanings, unless the context requires otherwise.

1.1 "2014 License Agreement" means the Exclusive Patent License Agreement between Company and Hospital dated the 29th of August, 2014.

1.2 "Affiliate" with respect to either Party shall mean any corporation or other legal entity other than that Party in whatever country organized, controlling, controlled by or under common control with that Party. The term "control" shall mean (i) in the case of Company, direct or indirect ownership of fifty percent (50%) or more of the voting securities having the right to elect directors, and (ii) in the case of Hospital, the power, direct or indirect, to elect or appoint fifty percent (50%) or more of the directors or trustees, or to cause direction of management and policies, whether through the ownership of voting securities, by contract or otherwise.

1.3 "Agriculture" shall mean (i) plants, fungi, and algae, including the microbiome for said plants, fungi and algae, propagated, cultivated or grown for food, material, clothing, livestock fodder, biofuel, ornamentals, medicine or other purpose and (ii) animals created, bred or raised for human consumption.

1.4 "Asset Sale" means the sale, lease, transfer or exclusive license of all or substantially all of the assets of Company to another entity that is not an Affiliate of Company.

1.5 "Average Market Capitalization" means the result of (i) sum of the Market Capitalizations on each Trading Day during a specified period of time divided by (ii) the number of Trading Days during such specified period of time.

1.6 "Claim" shall mean any pending or issued and unexpired claim of any Patent Right that has not been (i) permanently revoked, nor held unenforceable, unpatentable or invalid by a decision of a court or other governmental agency of competent jurisdiction that is unappealable, or unappealed in the time allowed for appeal, (ii) disclaimed or rendered unenforceable through disclaimer or otherwise, or (iii) abandoned.

1.7 "Common Stock" means the common stock, par value $0.0001 per share, of Company.

1.8 "Company Sale Date" means the date of closing of an Asset Sale or Merger.

1.9 "CRISPR" shall mean clustered regularly interspaced short palindromic repeats.

1.10 "Distributor" shall mean any third party entity to whom Company, a Company Affiliate or a Sublicensee has granted, express or implied, the right to distribute any Product or Process pursuant to Section 2.1(b)(ii).

1.11 "Election Date" shall have the meaning set forth in Section 4.6(d).

1.12 "Exchange Act" means the Securities and Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

1.13 "Expiration Date" shall have the meaning set forth in Section 10.1.

1.14 "FDA" shall mean the United States Food and Drug Administration or foreign equivalent.

1.15 "Field" shall mean the prevention and treatment of human diseases and the prevention and treatment of animal diseases. Specifically excluded from the Field are all use(s) of Product and/or Process for (i) clinical diagnostic assay and (ii) Agriculture.

1.16 "First Commercial Sale" shall mean the initial Sale anywhere in the applicable License Territory of a Product or Process after receipt of all applicable regulatory approvals, including pricing approvals, in the country in which such Product or Process is Sold.

1.17 "GAAP" means generally accepted accounting principles.

1.18 "IND" shall mean Investigational New Drug Application or foreign equivalent.

1.19 "License Territory" shall mean worldwide.

1.20 "Market Capitalization" means, with respect to a particular date, the [**] share price of Common Stock on such date multiplied by the number of shares of Common Stock outstanding used to calculate earnings per share in accordance with GAAP as set forth in Company's [**].

1.21 "Merger" means the merger or consolidation of Company with or into another entity (other than a merger with or into an Affiliate of Company or in which the pre-merger stockholders of Company own, immediately after such transaction, a majority of the total voting power represented by the outstanding voting securities of the surviving entity).

1.22 "Net Sales" shall be calculated as set forth in this Section 1.22.

(a) Subject to the conditions set forth below, "Net Sales" shall mean:

(i) the gross amount billed or invoiced, or if no such bill or invoice is issued the amount received, whichever is greatest, by Company and its Affiliates and Sublicensees for or on account of Sales of Products and Processes;

(ii) less the following amounts:

(A) to the extent actually allowed or paid as shown in documentation by Company, its Affiliates or its Sublicensees in effecting such Sale:

1. amounts repaid or credited by reason of rejection, return or recall of Products or Processes;

2. commercially reasonable trade, quantity or cash rebates or discounts to the extent taken;

3. commercially reasonable allowances for non-collectible receivables;

4. amounts for outbound transportation, insurance, packaging, handling and shipping, but only to the extent separately invoiced in a manner that clearly specifies the charges applicable to the applicable Products or Processes; and

5. taxes, customs duties and other governmental charges levied on or measured by production, Sale, transportation, or delivery of Products or Processes, to the extent separately stated on purchase orders, invoices or other documents of sale that are paid by or on behalf of

Company, its Affiliates or its Sublicensees, but not franchise or income taxes of any kind whatsoever.

(B) the gross amount billed or invoiced, or if no such bill or invoice is issued the amount received, whichever is greatest, by Company and its Affiliates and Sublicensees for or on account of Sales of Products and Processes to Hospital and Hospital's Affiliates.

(b) Specifically excluded from the definition of "Net Sales" are amounts attributable to any Sale of any Product or Process between or among Company and any Company Affiliate and/or Sublicensee, unless the transferee is the end purchaser, user or consumer of such Product or Process.

(c) Net Sales shall not include (a) sales or other transfers of any Product or Process used for clinical trials or other research on such Product or Process or (b) commercially reasonable donations of any Product or Process for charity or compassionate use for which Company or its Affiliate or Sublicensee making such donation does not receive consideration.

(d) No deductions shall be made for any commissions paid to any individuals or for any costs or expenses of collections.

(e) Net Sales shall be deemed to have occurred and the applicable Product or Process "Sold" on the date of billing or invoicing, or if no such bill or invoice is issued, the date of payment.

(f) If any Product or Process is Sold (i) in a product or transaction bundle that includes cash consideration that is not included in or provided for in the calculation of Net Sales with respect to such transaction and at a discounted price that is lower than the customary price charged or (ii) for non-cash consideration (whether or not at a discount), Net Sales shall be calculated based on the average non-discounted cash amount charged to independent third parties for the Product or Process during the same Reporting Period or, in the absence of such transactions, on the fair market value of the Product or Process assuming an arm's length transaction made in the ordinary course of business.

1.23 "NDA" shall mean a New Drug Application or foreign equivalent.

1.24 "Patent Rights" shall mean, inclusively, any patent or patent application listed in **Appendix A** and/or the equivalent of such application including any division, continuation, continuation-in-part (but only to the extent of claims directed to the subject matter claimed in the parent application), substitutes, counterparts and/or any foreign equivalents thereof filed in any country, Letters Patent, and/or the equivalent thereof issuing thereon, and/or reissue, reexamination or extension thereof.

1.25 "Payment Date" shall have the meaning set forth in Section 4.6(d).

1.26 "Principal Trading Market" means the Trading Market on which the Common Stock is primarily listed on and quoted for trading, which, as of the Effective Date is the NASDAQ Global Select Market.

1.27 "Process" shall mean any process, method or service the use or performance of which, in whole or in part:

(a) absent the license granted hereunder would infringe, or is covered by, one or more Claims of Patent Rights; or

(b) employs, incorporates, is based upon, or is derived from Technological Information.

1.28 "Product" shall mean any article, device or composition, the manufacture, use, or sale of which, in whole or in part:

(a) absent the license granted hereunder would infringe, or is covered by, one or more Claims of Patent Rights; or

(b) employs, incorporates, is based upon, or is derived from Technological Information.

1.29 "Public Company" means an issuer of Public Securities.

1.30 "Public Securities" means securities registered under the Securities Act that are listed on a national securities exchange registered under the Exchange Act or if not listed on a national securities exchange registered under the Exchange Act, quoted on NASDAQ, OTCQB or other similar quotation system.

1.31 "Record Retention Period" means [**] years following the end of the calendar year to which a record pertains.

1.32 "Reporting Period" shall mean each three-month period ending March 31, June 30, September 30 and December 31.

1.33 "Securities Act" means the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

1.34 "Sell" (and "Sale" and "Sold" as the case may be) shall mean to sell or have sold, to lease or have leased, to import or have imported, to export or have exported or otherwise to transfer or have transferred a Product or Process for valuable consideration (in the form of cash or otherwise), and further in the case of a Process to use or perform such Process for the benefit of a third party.

1.35 "Sublicensee" shall mean any sublicensee of rights granted in accordance with Section 2.1(a)(iii). For purpose of this Agreement, a Distributor of a Product or Process shall not be

included in the definition of Sublicensee unless such Distributor (i) is granted any right to make, have made, use or have used Products or Processes in accordance with Section 2.1(a)(iii), or (ii) has agreed to pay to Company or its Affiliate(s) royalties on such Distributor's sales of Products or Processes, in which case such Distributor shall be a Sublicensee for all purposes of Agreement.

1.36 "Subsequent Shares" shall have the meaning set forth in Section 4.6(d).

1.37 "Success Payment" shall have the meaning set forth in Section 4.6(b).

1.38 "Success Payment Period" means the period that [**].

1.39 "Technological Information" shall mean research data, designs, formulae, process information and other information pertaining to the invention(s) described in Patent Rights which is created by [**] and owned by Hospital and is not confidential information of, or otherwise obligated to, any third party and which [**] knows as of the Effective Date and reasonably believes is necessary in order for Company to utilize the licenses granted hereunder, as further described in **Appendix B**. Company agrees to treat all Technological Information in accordance with the provisions of **Appendix D.**

1.40 "Trading Day" means (i) a day on which the Common Stock is listed or quoted and traded on its Principal Trading Market (other than the OTC Bulletin Board), or (ii) if the Common Stock is not listed on a Trading Market (other than the OTC Bulletin Board), a day on which the Common Stock is traded in the over-the-counter market, as reported by the OTC Bulletin Board, or (iii) if the Common Stock is not quoted on any Trading Market, a day on which the Common Stock is quoted in the over-the-counter market as reported in the "pink sheets" by Pink Sheets LLC (or any similar organization or agency succeeding to its functions of reporting prices).

1.41 "Trading Market" means whichever of the New York Stock Exchange, the NYSE Amex Equities (formerly the American Stock Exchange), the NASDAQ Global Select Market, the NASDAQ Global Market, the NASDAQ Capital Market or the OTC Bulletin Board on which the Common Stock is listed or quoted for trading on the date in question.

1.42 "Trigger Date" means the [**].

1.43 "Value Trigger" shall have the meaning set forth in Section 4.6(b).

ARTICLE 2. LICENSE

2.1 Grant of License.

(a) Subject to the terms of Agreement and Hospital's rights in Patent Rights, Hospital hereby grants to Company in the License Territory:

(i) an exclusive, royalty-bearing license, sublicensable in accordance with Section 2.1(a)(iii), under Hospital's rights in Patent Rights to make, have

made, use, have used, Sell, offer for Sale and have Sold Products and/or Processes in the License Territory in the Field;

(ii) a non-exclusive, royalty-bearing license, sublicensable in accordance with Section 2.1(a)(iii), to use Technological Information to make, have made, use, have used, Sell, offer for Sale and have Sold Products and/or Processes in the License Territory in the Field; and

(iii) the right to grant sublicenses under the rights granted in Section 2.1(a)(i) and 2.1(a)(ii) to a Sublicensee, provided that in each case Company shall be responsible for the performance of any obligations of Sublicensee relevant to Agreement as if such performance were carried out by Company itself, including, without limitation, the payment of any royalties or other payments provided for hereunder, regardless of whether the terms of any sublicense provide for such amounts to be paid by the Sublicensee directly to Hospital.

(b) The license granted in Section 2.1(a) above includes:

(i) the right to grant to the final purchaser, user, or consumer of Product or Process the right to use such purchased Product or Process in a method coming within the scope of Patent Rights within the License Territory; and

(ii) the right to grant a Distributor the right to Sell (but not to make, have made, use or have used) such Product and/or Process for or on behalf of Company, its Affiliate or its Sublicensee in a manner consistent with Agreement.

(c) The foregoing license grant shall include the grant of such license to any Company Affiliate, provided that such Affiliate shall assume the same obligations as those of Company hereunder and be subject to the same terms and conditions hereunder; and further provided that Company shall be responsible for the performance of all of such obligations and for compliance with all of such terms and conditions by such Affiliate. Company shall provide to Hospital a fully signed, non-redacted copy of each agreement with each Affiliate that assumes the aforesaid obligations, including all exhibits, attachments and amendments and any related documents that alter, amend or otherwise modify such Affiliate's assumption of such obligations, within [**] days of request by Hospital.

2.2 Right to Subcontract. If Company desires to exercise any of the rights or obligations that Company may have under Agreement by subcontracting the exercise or performance of all or any portion of such rights and obligations on Company's behalf, Company shall be entitled to do so, provided that (a) such contract service providers obtain no rights in or to Patent Rights or Technological Information, (b) any subcontract granted or entered into by Company as contemplated by this Section 2 of the exercise or performance of all or any portion of the rights or obligations that Company may have under Agreement shall not relieve Company from any of

its obligations under Agreement, (c) any act or omission by a subcontractor of Company shall be deemed an act or omission by Company hereunder and (d) Company shall be responsible for each of its subcontractors complying with all obligations of Company under Agreement (including without limitation all restrictions placed on Company herein).

2.3 Sublicenses. Company may, without Hospital's prior written approval, enter into sublicense agreements, and Company shall provide to Hospital a fully signed non-redacted copy of all sublicense agreements and amendments thereto, including all exhibits, attachments and amendments, and any related documents that alter, amend or otherwise modify the rights or obligations of the Sublicensee under such sublicense agreement, within [**] days of executing the same; provided, however, that Company may redact from such copy (a) the identity of a genomic target selected for research, development or commercialization under the sublicense and (b) other proprietary non-public technical information of Company or Sublicensee. Each sublicense granted hereunder shall be consistent with and comply with all terms of Agreement, shall incorporate terms and conditions sufficient to enable Company to comply with Agreement, shall prohibit any further sublicense or assignment by a Sublicensee without Hospital's prior written consent (except that a Sublicensee may assign the applicable sublicense without Hospital consent to the same extent Company may assign Agreement under Section 12.5) and shall provide that Hospital is a third party beneficiary for the purpose of enforcing all patent challenge, indemnification and insurance provisions of such sublicense. Upon termination of Agreement or any license granted hereunder for any reason, any sublicenses shall be addressed in accordance with Section 10.8. Any sublicense which is not in accordance with the foregoing provisions shall be null and void. Hospital shall have the right to require Company to obtain Hospital's prior written approval for Company to enter all subsequent sublicenses if Hospital determines Company has materially failed to comply with the sublicensing provisions of Agreement and has not cured such non-compliance within [**] days after notice by Hospital.

2.4 Retained Rights; Requirements. Any and all licenses granted hereunder are subject to:

(a) the right of Hospital and Hospital's Affiliates and academic, government and not-for-profit institutions to make and to use the subject matter described and/or claimed in Patent Rights for research and educational purposes; and

(b) for Patent Rights supported by federal funding, the rights, conditions and limitations imposed by U.S. law (*see* 35 U.S.C. § 202 et seq. and regulations pertaining thereto), including without limitation:

(i) the royalty-free non-exclusive license granted to the U.S. government; and

(ii) the requirement that any Products used or sold in the United States shall be manufactured substantially in the United States.

2.5 No Additional Rights. It is understood that nothing in Agreement shall be construed to grant Company or any of its Affiliates a license, express or implied, under any patent owned solely or jointly by Hospital other than Patent Rights expressly licensed hereunder. Hospital

shall have the right to license any Patent Rights to any other party for any purpose outside of the Field or the License Territory.

2.6 Disclosure of Technological Information. At Company's request prior to execution of Agreement, Hospital (through [**]) shall use reasonable efforts to disclose in confidence within [**] days and not more than [**] days after execution of Agreement the Technological Information licensed hereunder.

ARTICLE 3. DUE DILIGENCE OBLIGATIONS

3.1 Diligence Requirements. Company shall use, and shall cause its Affiliates and Sublicensee, as applicable, to use, commercially reasonable efforts to research, develop and make available to the public Product and Process in the License Territory in the Field. Such efforts shall include achieving the following objectives within the time periods designated below following the Effective Date:

(a) within [**] months of Effective Date and within [**] days after the [**] thereafter commencing after the Effective Date until the [**] anniversary of the Effective Date and within [**] days after the [**] of the Effective Date, Company will [**]; provided that [**];

(b) within [**] years of Effective Date, Company will [**];

(c) within [**] years of Effective Date, Company will [**];

(d) within [**] years of Effective Date, Company will [**]; and

(e) within [**] years of Effective Date, Company will [**].

Achievement of the foregoing objectives shall be deemed to satisfy Company's obligations to use commercially reasonable efforts under this Section 3.1.

3.2 Diligence Failures. If Hospital determines that Company has failed to fulfill any of its obligations under Section 3.1, then Hospital may treat such failure as a default and may terminate Agreement and/or any license granted hereunder in accordance with Section 10.4.

3.3 Diligence Reports. Company shall provide all reports with respect to its obligations under Section 3.1 as set forth in Section 5.

ARTICLE 4. PAYMENTS, ROYALTIES, AND EQUITY

4.1 License Issue Fee. Company shall pay Hospital a non-refundable license issue fee in the amount of seven hundred fifty thousand dollars ($750,000) within [**] days after execution of Agreement.

4.2 Patent Cost Reimbursement. Company shall reimburse Hospital for all past patent costs and future reasonable, out-of-pocket costs associated with the preparation, filing, prosecution and maintenance of all Patent Rights ("Patent Costs"). As of the Effective Date, Hospital has

incurred approximately [**] dollars ($[**]) in Patent Costs, which amount Company shall pay to Hospital within [**] days after the Effective Date. Company shall pay to Hospital, or at Hospital's request directly to patent counsel, all other Patent Costs within [**] days of Company's receipt of an invoice for such Patent Costs either from Hospital or Hospital's patent counsel. Company agrees to indemnify, defend and hold Hospital harmless from and against any and all liabilities, damages, costs and expenses arising from the failure of Company to timely pay such invoices and Patent Costs. Hospital shall instruct patent counsel to provide copies to Hospital for Hospital's administrative files of all invoices detailing Patent Costs which are sent directly to Company. If Company pays any Patent Costs directly, Company shall advise patent counsel that Hospital is and shall remain patent counsel's client.

4.3 Annual License Fee; Annual Minimum Royalty. Company shall pay to Hospital a non-refundable annual license fee of (a) [**] dollars ($[**]) on the second anniversary of the Effective Date and each subsequent anniversary of the Effective Date until and including the [**] anniversary of the Effective Date and (b) [**] dollars ($[**]) on the [**] anniversary of the Effective Date and each subsequent anniversary of the Effective Date until the Expiration Date. Each annual license fee set forth in this Section 4.3 is creditable against royalties payable under Section 4.5 in the same calendar year in which the anniversary of the Effective Date giving rise to the payment of such annual license fee occurs.

4.4 Milestone Payments. In addition to the payments set forth in Sections 4.1 through 4.3 above, Company shall pay Hospital milestone payments within the scope of Field within [**] days after achievement of the following milestones:

(a) a one-time payment of [**] dollars ($[**]) upon the [**];

(b) a one-time payment of [**] dollars ($[**]) upon the [**];

(c) [**] dollars ($[**]) upon the [**];

(d) [**] dollars ($[**]) upon the [**];

(e) [**] dollars ($[**]) upon [**] and [**] dollars ($[**]) upon [**];

(f) [**] dollars ($[**]) upon [**] and [**] dollars ($[**]) upon [**];

(g) a one-time payment of [**] dollars ($[**]) when total Net Sales of Product or Process in any calendar year reach [**] dollars ($[**]);

(h) a one-time payment of [**] dollars ($[**]) when total Net Sales of Product or Process in any calendar year reach [**] dollars ($[**]); and

(i) a one-time payment of [**] dollars ($[**]) when total Net Sales of Product or Process in any calendar year reach [**] dollars ($[**]).

No payment will be due on a replacement Product or Process for a previously achieved milestone set forth above in this Section 4.4. Except as expressly set forth above, each milestone is payable only once.

For purposes of the milestones set forth above in this Section 4.4, the term [**]" shall exclude any [**].

Any of the foregoing milestones is only payable if the applicable Product and/or Process is covered by a Claim at the time of the achievement of such milestone.

4.5 Royalties.

(a) Beginning with the First Commercial Sale in any country in the License Territory, Company shall pay Hospital royalties on Net Sales of Products and Processes on a Product/Process-by-Product/Process and country-by-country basis as follows:

(i) a royalty of [**] percent ([**]%) of the Net Sales by Company, a Company Affiliate, or a Company Sublicensee of any Product and/or Process for the prevention and treatment of human disease that is covered by a Claim;

(ii) If [**] such Product and/or Process, a royalty of [**] percent ([**]%) of the Net Sales by Company, a Company Affiliate, or a Company Sublicensee of such Product and/or Process [**];

(iii) a royalty of [**] percent ([**]%) of the Net Sales by Company, a Company Affiliate, or a Company Sublicensee of any Product and/or Process [**]; and

(iv) If [**] covering such Product and/or Process, a royalty of [**] percent ([**]%) of the Net Sales by Company, a Company Affiliate, or a Company Sublicensee of such Product and/or Process [**].

(b) In the event that a Product and/or Process is (i) covered by a "Claim" or employs "Tangible Materials" or "Technological Information" (each as defined under the 2014 Agreement) and (ii) covered by a Claim under this Agreement, Company may deduct up to [**] percent ([**]%) of royalties Company, Company Affiliate, or Company Sublicensee pays to third parties for Product and/or Process covered under Sections 4.5(a)(i)-(iv) of this Agreement from the respective royalty due to Hospital under Sections 4.5(a)(i)-(iv) of this Agreement, but the total reduction of each royalty under Sections 4.5(a)(i)-(iv) of this Agreement will not exceed [**] percent ([**]%). Provided, however, in the event that a Product and/or Process is (i) not covered by "Claim" or employing "Tangible Materials" or "Technological Information" (each as defined under the 2014 Agreement) and (ii) is covered by a Claim under this Agreement, Company may not deduct any royalties Company,

Company Affiliate or Company Sublicensee pays to a third party from the respective royalty due to Hospital under this Agreement.

(c) Only one royalty under this Agreement shall be due on any Sale of a Product or Process no matter how many Claims cover such Product or Process and no matter how many provisions of Section 4.5(a) apply to such Product or Process. In the event more than one provision of Section 4.5(a) would apply to any Product or Process, only the highest applicable royalty shall be payable on any Sale of such Product or Process.

(d) Royalties shall be due on a country-by-country and Product/Process-by-Product/Process basis ending on the later of the following:

(i) the expiration of the last Claim within Patent Rights covering the applicable Product and/or Process; and

(ii) the tenth anniversary of the date of First Commercial Sale of the applicable Product and/or Process.

(e) Upon expiration of the obligation to pay royalties on a Product/Process in a country in accordance with Section 4.5(d), the licenses granted hereunder with respect to such Product/Process in such country shall become perpetual, irrevocable, fully paid up, sublicensable licenses.

(f) All payments due to Hospital under this Section 4.5 shall be due and payable by Company within [**] days after the end of each Reporting Period, and shall be accompanied by a report as set forth in Sections 5.3 and 5.4.

4.6 Success Payments.

(a) Notice. Company shall notify Hospital within [**] days after any Trigger Date. Such notice shall include the date of such Trigger Date and a determination of the Average Market Capitalization as of such Trigger Date.

(b) Achievement of Average Market Capitalization. If on a Trigger Date during the Success Payment Period, the Average Market Capitalization as of such Trigger Date is for the first time as of any Trigger Date equal to or in excess of an amount shown in the column below labeled “Value Trigger” (each such amount, a “Value Trigger”), Company shall pay to Hospital such payment indicated opposite such Value Trigger in the column labeled “Success Payment” (each such payment, a “Success Payment”):

Value Trigger	Success Payment
$[**]	$[**]
$[**]	$[**]
$[**]	$[**]
$[**]	$[**]

The $[**] and $[**] Success Payments apply if on a Trigger Date a Product covered by a Claim (i) is the subject of a Phase I clinical study of which the Company or its Affiliate or Sublicensee is the sponsor, (ii) in the case of a Product that has completed a Phase I clinical study, the Company is conducting, or has determined to conduct, a subsequent clinical study with respect to such Product, or (iii) has been approved by the relevant regulatory authority for sale in either the United States or the European Union. If either Value Trigger is achieved and (i), (ii), or (iii) is met after such Value Trigger and Market Capitalization is still at or above such Value Trigger, the applicable Success Payment shall apply upon fulfillment of (i), (ii), or (iii).

For the avoidance of doubt, each Success Payment shall become due and payable under Agreement, if at all, a maximum of one (1) time. For the further avoidance of doubt, more than one Success Payment may become due and payable based on the Average Market Capitalization determined on any single Trigger Date. By way of example under the immediately preceding sentence, if the Average Market Capitalization on the first Trigger Date that is more than [**] days after the Effective Date is $[**], then Company shall pay to Hospital aggregate Success Payments equal to $[**] if no Success Payments have been paid previously by Company.

(c) Company Sale Success Payment. Notwithstanding anything to the contrary herein, if a Trigger Date during the Success Payment Period is a Company Sale Date, the Company shall pay to Hospital the amount equal to the sum of all Success Payments that (i) correspond to the Value Triggers that are lower than or equal the total consideration paid (regardless of whether such consideration is paid in cash, stock, by assumption of debt or otherwise) by the acquirer (or its successors or assigns, as applicable) by the Company Sale Date, inclusive of all applicable Deductions, but exclusive of the consideration paid by the acquirer (or its successors or assigns, as applicable) after the Company Sale Date (including without limitation contingent payments, royalties, earn-outs or milestone payments) and (ii) are unpaid as of the day immediately prior to the Company Sale Date.

(d) Manner and Timing of Payment. Any Success Payment provided herein that is payable with respect to a Trigger Date that is not a Company Sale Date, will be paid by Company in cash or in shares of Common Stock (any such shares, "Subsequent Shares"), and the form of such payment, cash or Subsequent Shares, shall be determined solely by Company. Company shall notify Hospital of its election with regard to the form of payment of a Success Payment within [**] days after the applicable Trigger Date (the "Election Date"). Any Success Payment provided herein that is payable with respect to a Trigger Date that is a Company Sale Date, will be paid by Company solely in cash. Success Payments

shall be made no later than [**] days after the applicable Trigger Date (the "Payment Date"); provided, however, that if Company is a Public Company and elects to pay a Success Payment in shares of Common Stock, then the following shall apply:

[**].

(e) Calculation of Number of Subsequent Shares. If Company elects to pay a Success Payment in shares of Common Stock as provided in Section 4.6(d), the number of such Subsequent Shares shall equal the applicable Success Payment divided by [**].

(f) Restrictions on Sales. Notwithstanding anything to the contrary in Agreement, Hospital agrees with respect to Subsequent Shares received by Hospital that prior to the date that is [**] days after the date of receipt of such Subsequent Shares, Hospital will not offer, sell or otherwise dispose of more than [**] ([**]%) of such Subsequent Shares during each of the following periods: [**] day thereafter. The restrictions on sale provided in immediately preceding sentence shall not be applicable to an offer, sale or other disposition in connection with and as part of a merger, consolidation, business combination, recapitalization, liquidation, dissolution or similar transaction involving Company pursuant to which the stockholders of Company immediately preceding such transaction will hold less than [**] percent ([**]%) of the aggregate equity interests in the surviving or resulting entity of such transaction or any direct or indirect parent thereof.

4.7 Form of Payment. Except as set forth in Section 4.6, all payments due under Agreement shall be drawn on a United States bank and shall be payable in United States dollars. Each payment shall reference Agreement and its Agreement Number and identify the obligation under Agreement that the payment satisfies. Conversion of foreign currency to U.S. dollars shall be made at the conversion rate existing in the United States, as reported in The Wall Street Journal, or, solely with respect to Sublicensees, at another commercially reasonable, publicly available, applicable conversion rate as may be provided in a sublicense, on the last working day of the applicable Reporting Period. Such payments shall be without deduction of exchange, collection or other charges, and, specifically, without deduction of withholding or similar taxes or other government imposed fees or taxes imposed on Company, except as permitted in the definition of Net Sales.

Checks for all payments due to Hospital under Agreement shall be made payable to Hospital and addressed as set forth below:

Massachusetts General Hospital
BOA-Lockbox Services
PCSR Lockbox #415007
MA5-527-02-07
2 Morrissey Blvd
Dorchester, MA 02125

Reference Agreement #: A224596

Payments via wire transfer should be made as follows:

ACH Credit: [**]
Federal Reserve Wire: [**]
SWIFT Code: [**]
Account #[**]
Massachusetts General Hospital
[**]

Reference Agreement #: A224596

4.8 Overdue Payments. The payments due under Agreement shall, if overdue, bear interest beginning on the first day following the Reporting Period to which such payment was incurred and until payment thereof at a per annum rate equal to [**] percent ([**]%) above the prime rate in effect on the due date as reported by The Wall Street Journal, such interest rate being compounded on the last day of each Reporting Period, not to exceed the maximum permitted by law. Any such overdue payments when made shall be accompanied by all interest so accrued. Said interest and the payment and acceptance thereof shall not preclude Hospital from exercising any other rights it may have as a consequence of the lateness of any payment.

ARTICLE 5. REPORTS AND RECORDS

5.1 Diligence Reports. Within [**] days after the end of each [**], Company shall report in writing to Hospital on progress made toward the objectives set forth in Section 3.1 during such preceding [**] month period, including, without limitation, progress on research and development, status of applications for regulatory approvals, manufacturing, sublicensing and the number of sublicenses entered into and marketing.

5.2 Milestone Achievement Notification. Company shall report to Hospital the dates on which it achieves the milestones set forth in Section 4.4 within [**] days of each such occurrence.

5.3 Sales Reports. Company shall report to Hospital the date of the First Commercial Sale in each country of the License Territory within [**] days of each such occurrence. Following the First Commercial Sale, Company shall deliver to Hospital, within [**] days after the end of each Reporting Period, a report under this Section 5.3 substantially in the format outlined in **Appendix C**, which report shall be certified as correct by an officer of Company and shall contain at least the following information as may be pertinent to a royalty accounting hereunder for the immediately preceding Reporting Period:

(a) the number of Products and Processes Sold by Company, its Affiliates and Sublicensees in each country;

(b) the amounts billed or invoiced, or if no bill or invoice, received, by Company, its Affiliates and Sublicensees for each category or class of Product and Process, in each country, and total billings or payments due or made for all Products and Processes;

(c) calculation of Net Sales for the applicable Reporting Period in each country, including an itemized listing of permitted offsets and deductions (provided that in the case of sublicenses, this obligation shall apply only to the extent such itemized listing of permitted offsets and deductions is available from a Sublicensee under the terms of the relevant sublicense);

(d) total royalties payable on Net Sales in U.S. dollars, together with the exchange rates used for conversion; and

(e) any other payments due to Hospital under Agreement.

If no amounts are due to Hospital for any Reporting Period, the report shall so state.

5.4 Audit Rights.

(a) During the Record Retention Period, Company shall maintain, and shall cause each of its Affiliates and Sublicensees to maintain, complete and accurate records relating to the rights and obligations under Agreement and any amounts payable to Hospital in relation to Agreement, which records shall contain sufficient information to permit Hospital and its representatives to confirm the accuracy of any payments and reports delivered to Hospital and compliance in all other respects with Agreement. Company shall retain and make available, and shall cause each of its Affiliates and Sublicensees to retain and make available, such records during the Record Retention Period, to an independent, certified public accountant chosen by Hospital and reasonably acceptable to Company upon at least [**] days' advance written notice, for inspection during normal business hours, to verify any reports and payments made and/or compliance in other respects under Agreement. Such accountant shall not disclose to Hospital any information other than information relating to the accuracy of reports and payments delivered under Agreement. Notwithstanding the foregoing to the contrary, Hospital may not cause an audit of any Sublicensee unless Company has not conducted previously an audit of the relevant Reporting Period and fails or refuses to conduct such audit upon the reasonable written request of Hospital. If Company has conducted previously an audit of the relevant Reporting Period, Company shall make the results of such audit available to the independent, certified public accountant chosen by Hospital and reasonably acceptable to Company. If any audit conducted pursuant to the provisions of this Section 5.4(a) shows an underreporting or underpayment of [**] percent ([**]%) or more in any payment due to Hospital hereunder, Company shall bear the full cost of such audit and shall remit any amounts due to Hospital (including interest due in accordance

with Section 4.8) within [**] days of receiving notice thereof from Hospital. Hospital may exercise its rights under this Section 5.4(a) only [**] and only [**].

(b) During the Record Retention Period, Hospital shall cause each of its patent counsel to maintain complete and accurate records relating to the Patent Costs, which records shall contain sufficient information to permit Company and its representatives to confirm the accuracy of any requests for reimbursement or direct payment by Company of the Patent Costs. Hospital shall cause each of its patent counsel to retain and make available, and shall cause each of its patent counsels to retain and make available, such records during the Record Retention Period, to an independent, certified public accountant chosen by Company and reasonably acceptable to Hospital upon at least [**] days' advance written notice, for inspection during normal business hours, to verify any reports and payments made. Such accountant shall not disclose to Company any information other than information relating to the accuracy of the Patent Costs and related invoices and requests for payment. If any audit conducted pursuant to the provisions of this Section 5.4(b) shows an overpayment of Patent Costs by Company, Hospital shall credit Company, or in the case of direct payment by Company to Hospital's patent counsel have Hospital's patent counsel credit Company, for all such overpayment of Patent Costs in the next payment of Patent Costs due hereunder and any subsequent payment of Patent Costs due hereunder until said credit is fully applied. Company may exercise its rights under this Section 5.4(b) only [**] and only [**].

ARTICLE 6. PATENT PROSECUTION AND MAINTENANCE

6.1 Prosecution. Hospital shall be responsible for the preparation, filing, prosecution and maintenance of all patent applications and patents included in Patent Rights. Company will have sufficient rights to influence the prosecution of Patent Rights within the scope of Field. With respect to national stage entry of a patent application, Company shall provide Hospital with a list of countries in which Company would like Hospital to file patent applications. Hospital shall file, prosecute and maintain such patent applications and resulting patents in all jurisdictions requested by Company. If with respect to any patent application, Hospital wishes to file patent applications in additional countries not requested by Company, Hospital shall notify Company, and Company and Hospital shall discuss the commercial value of filing such patent applications in such additional countries. If Company does not agree in writing to the filing of such patent applications in such additional countries within [**] days from said notification, (i) all costs incurred by Hospital in connection with the preparation, filing, prosecution and maintenance of such patent applications in such additional countries shall be excluded from Patent Costs for which Company shall pay or reimburse hereunder, (ii) Hospital may file such patent applications in such additional countries at its own expense, and (iii) such patent applications filed by Hospital in such countries shall not be considered in Patent Rights. Company shall reimburse Hospital for Patent Costs incurred by Hospital relating thereto in accordance with Section 4.2.

6.2 Copies of Documents. With respect to any Patent Right licensed hereunder, Hospital shall instruct the patent counsel prosecuting such Patent Right to (i) copy Company on patent

prosecution documents that are received from or filed with the United States Patent and Trademark Office and foreign equivalent, as applicable; (ii) if requested by Company, provide Company with copies of draft applications and other submissions to any patent office prior to filing; and (iii) give due consideration to the comments and requests of Company or its patent counsel, which Hospital and its patent counsel will not unreasonably refuse to incorporate or address.

6.3 Company's Election Not to Proceed. Company may elect to surrender any patent or patent application in Patent Rights in any country upon [**] days advance written notice to Hospital. Such notice shall relieve Company from the obligation to pay for future Patent Costs but shall not relieve Company from responsibility to pay Patent Costs incurred prior to the expiration of the [**] day notice period. Such U.S. or foreign patent application or patent shall thereupon cease to be a Patent Right hereunder, Company shall have no further rights therein and Hospital shall be free to license its rights to that particular U.S. or foreign patent application or patent to any other party on any terms.

6.4 Patent Term Extensions. Company shall have the exclusive right to seek patent term extensions or supplemental patent protection, including supplementary protection certificates, in any country in the License Territory in relation to Products and Processes in the Field at Company's expense. Hospital shall cooperate with Company in connection with all such activities. Hospital will promptly provide any instruments, agreements or other documents reasonably requested by Company in connection with any patent term extension or supplemental patent protection sought by Company that relates to a Patent Right.

6.5 Confidentiality of Prosecution and Maintenance Information. Company agrees to treat all information related to prosecution and maintenance of Patent Rights as Confidential Information (as defined in **Appendix D**) in accordance with the provisions of **Appendix D**. In addition, Company and Hospital acknowledge and agree that, with regard to filing, prosecution and maintenance of Patent Rights, the interests of the Parties as licensor and licensee are aligned and are legal in nature. The Parties agree and acknowledge that they have not waived, and nothing in Agreement constitutes a waiver of, any legal privilege concerning Patent Rights or a Party's Confidential Information, including privilege under the common interest doctrine and similar or related doctrines.

ARTICLE 7. THIRD PARTY INFRINGEMENT AND LEGAL ACTIONS

7.1 Hospital Right to Prosecute. Hospital will protect its Patent Rights from infringement and prosecute accused infringers when, in its sole judgment, such action may be reasonably necessary, proper and justified, except Company will have the sole right to enforce Patent Rights against accused infringers within the scope of Field.

7.2 Company Right to Prosecute. In accord with Section 7.1, Company may, upon notice to Hospital, initiate legal proceedings against an accused infringer at Company's expense with respect to a claim of a Patent Right in the License Territory. Before commencing such action, Company and, as applicable, any Affiliate, shall consult with Hospital, concerning, among other things, Company's standing to bring suit, the advisability of bringing suit, the selection of

counsel and the jurisdiction for such action (provided Company must have Hospital's prior written consent with respect to selection of jurisdiction for any action in which Hospital may be joined as a party-plaintiff) and shall use reasonable efforts to accommodate the views of Hospital regarding the proposed action, including without limitation with respect to potential effects on the public interest. Company shall be responsible for all costs, expenses and liabilities in connection with any such action and shall indemnify and hold Hospital harmless therefrom, regardless of whether Hospital is a party-plaintiff, except for the expense of any independent counsel retained by Hospital in accordance with Section 7.5 below.

7.3 Hospital Joined as Party-Plaintiff. If Company elects to commence an action as described in Section 7.2 above, Hospital shall have, in its sole discretion, the option to join such action as a party-plaintiff. If Hospital is required by law to join such action as a party-plaintiff, Hospital may either, in its sole discretion, permit itself to be joined as a party-plaintiff at the sole expense of Company, or assign to Company all of Hospital's right, title and interest in and to the Patent Right which is the subject of such action (subject to all of Hospital's obligations to the government under law and any other rights that others may have in such Patent Right). If Hospital makes such an assignment, such action by Company shall thereafter be brought or continued without Hospital as a party (unless Hospital remains a necessary party as found by the relevant court or tribunal); provided, however, that Hospital and Company shall enter into a separate agreement providing Hospital with continuing rights of prosecution and maintenance of and requiring Company to continue to meet all of its obligations with respect to prosecution and maintenance of Patent Rights as if the assigned Patent Right were still licensed to Company hereunder.

7.4 Notice of Actions; Settlement. Company shall promptly inform Hospital of any action or suit relating to Patent Rights and shall not enter into any settlement, consent judgment or other voluntary final disposition of any action relating to Patent Rights, including but not limited to appeals, that admits liability, wrongdoing or fault by Hospital without the prior written consent of Hospital, which consent shall not be unreasonably withheld, conditioned or delayed.

7.5 Cooperation. Each Party agrees to cooperate reasonably in any action under Section 7 which is controlled by the other Party, provided that the controlling party reimburses the cooperating party for any reasonable costs and expenses incurred by the cooperating party in connection with providing such assistance, except for the expense of any independent counsel retained by the cooperating party in accordance with this Section 7.5. Such controlling party shall keep the cooperating party informed of the progress of such proceedings and shall make its counsel available to the cooperating party. The cooperating party shall also be entitled to independent counsel in such proceedings but at its own expense, said expense to be offset against any damages received by the Party bringing suit in accordance with Section 7.6 only if representation of the cooperating Party by counsel to the Party bringing suit would be inappropriate because of conflict of interests.

7.6 Recovery. Any award paid by third parties as the result of such proceedings (whether by way of settlement or otherwise) shall first be applied to reimbursement of any legal fees and expenses incurred by the Party bringing such proceeding and by the other Party if representation of such other Party by counsel to the Party bringing such proceeding would be inappropriate

because of conflict of interests and then the remainder shall be divided between the Parties for any proceedings related to the Field as follows:

(a) Company shall receive an amount equal to its lost profits or a reasonable royalty on the infringing sales, or whichever measure of damages the court shall have applied; and

(b) Hospital shall receive an amount equal to the royalties and other amounts that Company would have paid to Hospital if Company had Sold the infringing Products and Services rather than the infringer, provided that the amounts payable under this clause (ii) shall in no event exceed the amounts payable under clause (i) above; and

(c) the balance, if any, remaining after Company and Hospital have been compensated under Section 7.6(a) and (b) that is attributable to the infringement of Patent Rights shall be shared by the Parties as follows: [**] percent ([**]%) to Company and [**] percent ([**]%) to Hospital if Company brought and prosecuted such proceedings and [**] percent ([**]%) to Company and [**] percent ([**]%) to Hospital if Hospital brought and prosecuted such proceedings.

7.7 Patent Validity Challenge by a Third Party. Each Party shall promptly notify the other in the event it receives notice of any legal or administrative action by any third party against a Patent Right, including any oppositions, interference, derivation, revocation, reexamination, *inter partes* review, post-grant review, nullity action, compulsory license proceeding, or declaratory judgment action. Except as provided in the following sentence, opposition, interference and derivation proceedings shall be addressed as provided in Section 7.1. Company shall have the first right to defend in all revocation, reexamination, *inter partes* review, post-grant review, nullity action, compulsory licensing proceeding, or declaratory judgment actions as provided in Section 7.2. If Company elects not to participate in such action, it shall promptly notify Hospital in writing of its decision not to proceed and Hospital may elect to take over the defense at its own expense. Hospital shall give due consideration to Company's reasons for not participating or initiating in such action, which reasons will not be unreasonably disregarded, prior to initiating the defense of such action.

7.8 Third Party Patent Oppositions and Other Proceedings. If Hospital desires to bring an opposition, action for declaratory judgment, nullity action, interference, *inter partes* review, post-grant review or other action to challenge the validity, title, enforceability of a patent owned or controlled by a third party that covers or may cover the composition, manufacture, use or commercial sale of any Product or Process in the Field, Hospital shall first consult with Company prior to initiating such action. The Parties shall discuss in good faith the rationale for, and the proposed actions to be taken, with respect to such opposition or other action. Company shall have the first right, but not the obligation to take action. Hospital shall give due consideration to Company's reasons for not initiating such action, which will not be unreasonably disregarded, prior to initiating such action.

ARTICLE 8. INDEMNIFICATION AND INSURANCE

8.1 Indemnification.

(a) Company shall indemnify, defend and hold harmless Hospital and its Affiliates and their respective trustees, directors, officers, medical and professional staff, employees, and agents and their respective successors, heirs and assigns (the "Indemnitees"), against any liability, damage, loss or expense (including reasonable attorney's fees and expenses of litigation) incurred by or imposed upon the Indemnitees or any one of them in connection with any claims, suits, actions, demands or judgments (each an "Action") arising out of (i) any theory of product liability (including, but not limited to, actions in the form of contract, tort, warranty, or strict liability) concerning any product, process or service made, used, or sold or performed pursuant to any right or license granted under Agreement (ii) the practice of Company, its Affiliates or Sublicensees of any Patent Rights and/or rights granted in this Agreement or (iii) Company's breach of this Agreement, except to the extent any such Action results directly from the gross negligence or willful misconduct of an Indemnitee.

(b) Hospital agrees to provide Company with prompt written notice of any claim for which indemnification is sought under Agreement. Company agrees, at its own expense, to provide attorneys reasonably acceptable to Hospital to defend against any Action brought, filed against, or served upon any Indemnitee with respect to the subject of indemnity contained herein, whether or not such Action is rightfully brought; provided, however, that any Indemnitee shall have the right to retain its own counsel, at the expense of Company, if representation of such Indemnitee by counsel retained by Company would be inappropriate because of conflict of interests of such Indemnitee and any other party represented by such counsel. If applicable, Company agrees to keep Hospital informed of the progress in the defense and disposition of any Action and to consult with Hospital prior to any proposed settlement. If applicable, Hospital may not settle any Action for which it is claiming, or may in the future may make a claim for indemnification, hereunder without the prior written consent of Company.

(c) This Section 8.1 shall survive expiration or termination of Agreement.

8.2 Insurance.

(a) Beginning at such time as any such product, process or service is being commercially distributed, sold, leased or otherwise transferred, or performed or used (other than for the purpose of obtaining regulatory approvals), by Company, an Affiliate or Sublicensee, Company shall, at its sole cost and expense, procure and maintain commercial general liability insurance in amounts not less than $[**] per incident and $[**] annual aggregate and naming the Indemnitees as additional insureds. Such commercial general liability insurance shall provide (i) product liability coverage and (ii) broad form contractual liability coverage for

Company's indemnification under Section 8.1 of Agreement. If Company elects to self-insure all or part of the limits described above (including deductibles or retentions which are in excess of $[**] annual aggregate) such self-insurance program must be acceptable to Hospital and the Risk Management Foundation. The minimum amounts of insurance coverage required under this Section 8.2 shall not be construed to create a limit of Company's liability with respect to its indemnification under Section 8.1 of Agreement.

(b) Company shall provide Hospital with written evidence of such insurance upon request of Hospital. Company shall provide Hospital with written notice at least [**] days prior to the cancellation, non-renewal or material change in such insurance; if Company does not obtain replacement insurance providing comparable coverage prior to the expiration of such [**] day period, Hospital shall have the right to terminate Agreement effective at the end of such [**] day period without notice or any additional waiting periods.

(c) Company shall maintain such commercial general liability insurance beyond the expiration or termination of Agreement during (i) the period that any such product, process, or service is being commercially distributed, sold, leased or otherwise transferred, or performed or used (other than for the purpose of obtaining regulatory approvals), by Company or by a licensee, affiliate or agent of Company and (ii) a reasonable period after the period referred to in clause (i) above which in no event shall be less than [**] years.

(d) This Section 8.2 shall survive expiration or termination of Agreement.

ARTICLE 9. DISCLAIMER OF WARRANTIES; LIMITATION OF LIABILITY

9.1 Title to Patent Rights. To the best knowledge of Partners Healthcare Innovation, Hospital is the owner by assignment from [**] of Patent Rights and has the authority to enter into Agreement and license Patent Rights to Company hereunder.

9.2 No Warranties. HOSPITAL MAKES NO REPRESENTATIONS OR WARRANTIES OF ANY KIND, EXPRESS OR IMPLIED, CONCERNING PATENT RIGHTS AND THE RIGHTS GRANTED HEREUNDER, INCLUDING, WITHOUT LIMITATION, WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, NONINFRINGEMENT, VALIDITY OF PATENT RIGHTS CLAIMS, WHETHER ISSUED OR PENDING, AND THE ABSENCE OF LATENT OR OTHER DEFECTS, WHETHER OR NOT DISCOVERABLE, AND HEREBY DISCLAIMS THE SAME. SPECIFICALLY, AND NOT TO LIMIT THE FOREGOING, HOSPITAL MAKES NO WARRANTY OR REPRESENTATION (i) REGARDING THE VALIDITY OR SCOPE OF ANY OF THE CLAIM(S), WHETHER ISSUED OR PENDING, OF ANY OF PATENT RIGHTS, AND (ii) THAT THE EXPLOITATION OF PATENT RIGHTS OR ANY PRODUCT WILL NOT INFRINGE ANY PATENTS OR OTHER INTELLECTUAL PROPERTY RIGHTS OF HOSPITAL OR OF ANY THIRD PARTY.

9.3 Limitation of Liability. IN NO EVENT SHALL HOSPITAL OR ANY OF ITS AFFILIATES OR ANY OF THEIR RESPECTIVE TRUSTEES, DIRECTORS, OFFICERS, MEDICAL OR PROFESSIONAL STAFF, EMPLOYEES AND AGENTS BE LIABLE TO LICENSEE OR ANY OF ITS AFFILIATES, SUBLICENSEES OR DISTRIBUTORS FOR INDIRECT, SPECIAL, INCIDENTAL OR CONSEQUENTIAL DAMAGES OF ANY KIND ARISING IN ANY WAY OUT OF AGREEMENT OR THE LICENSE OR RIGHTS GRANTED HEREUNDER, HOWEVER CAUSED AND ON ANY THEORY OF LIABILITY, INCLUDING WITHOUT LIMITATION ECONOMIC DAMAGES OR INJURY TO PROPERTY OR LOST PROFITS, REGARDLESS OF WHETHER HOSPITAL SHALL BE ADVISED, SHALL HAVE OTHER REASON TO KNOW, OR IN FACT SHALL KNOW OF THE POSSIBILITY OF THE FOREGOING.

ARTICLE 10. TERM AND TERMINATION

10.1 Term. The term of Agreement shall commence on the Effective Date and shall remain in effect until the date on which there are no more pending or issued and unexpired Claims within Patent Rights ("Expiration Date"), unless Agreement is terminated earlier in accordance with any of the other provisions of this Article 10. Only upon Expiration Date and all payments from Company to Hospital have been made as required by Agreement, Company shall have a worldwide, perpetual, irrevocable, fully paid up, freely sublicensable license under the rights and licenses granted to Company under Section 2.1; provided, however, that the obligation of Company to pay royalties on Net Sales of Products and Processes for which the royalty term has not expired in accordance with Section 4.5(e) at Expiration Date shall continue uninterrupted until such expiration of Agreement in accordance with Section 4.5(e).

10.2 Termination for Failure to Pay. If Company fails to make any payment due hereunder, Hospital shall have the right to terminate Agreement upon [**] business days written notice, unless Company makes such payments within said [**] day notice period. If payments are not made, Hospital may immediately terminate Agreement at the end of said [**] day period.

10.3 Termination for Insurance and Insolvency.

(a) Insurance. Hospital shall have the right to terminate Agreement in accordance with Section 8.2(b) if Company fails to maintain the insurance required by Section 8.2.

(b) Insolvency and other Bankruptcy Related Events. Hospital shall have the right to terminate Agreement immediately upon written notice to Company with no further notice obligation or opportunity to cure if Company: (i) shall become insolvent; (ii) shall make an assignment for the benefit of creditors; (iii) shall file a petition in bankruptcy; or (iv) or shall have a petition in bankruptcy filed against it which shall remain undismissed and unstayed for a period of [**] days.

10.4 Termination for Non-Financial Default. If Company, any of its Affiliates or any Sublicensee shall default in the performance of any of its other material obligations under Agreement not otherwise covered by the provisions of Section 10.2 and 10.3, and if such default

has not been cured within [**] days after notice by Hospital in writing of such default, Hospital may immediately terminate Agreement, and/or any license granted hereunder with respect to the country or countries in which such default has occurred, at the end of said [**] day cure period.

10.5 Challenging Validity. During the term of Agreement, Company shall not challenge, and shall restrict Company Affiliates and Sublicensees from challenging, the validity of Patent Rights and in the event of any breach of this provision Hospital shall have the right to terminate Agreement and any license granted hereunder immediately. In addition, if Patent Rights are upheld Company shall reimburse Hospital for its legal costs and expenses incurred in defending any such challenge. Notwithstanding the foregoing to the contrary, if a Sublicensee is the party so challenging the validity of Patent Rights, Hospital may immediately terminate the rights hereunder only as and to the extent sublicensed to such Sublicensee. For clarity, in the case of any such termination of rights hereunder as and to the extent sublicensed to a Sublicensee, such termination shall not affect the rights hereunder held by Company or any other sublicense, and Agreement and such other sublicenses shall remain in full force and effect.

10.6 Termination by Company. Company shall have the right to terminate Agreement by giving ninety (90) days advance written notice to Hospital and upon such termination shall immediately cease all use and Sales of Products and Processes, subject to Section 10.10.

10.7 Special Provisions Regarding Breaches by Sublicensees. Notwithstanding anything in this Article 10 to the contrary, if a breach by Company under Section 10.2, 10.3 or 10.4 arises as a result of a breach by a Sublicensee of the terms of a sublicense and Company is using commercially reasonable efforts to cure such breach or terminate such sublicense, Hospital may not terminate Agreement during the pendency of such efforts or thereafter if such breach by such Sublicensee is cured or the relevant sublicense is terminated. If Company has used commercially reasonable efforts to cure such breach or terminate such sublicense but has not been able to cure such breach or terminate such sublicense within [**] days after receiving the first written notice of termination from Hospital relating to such breach hereunder, Hospital may not terminate Agreement but may terminate the rights hereunder as and to the extent sublicensed to such Sublicensee. For clarity, any such termination shall not affect the rights hereunder held by Company or any other Sublicense, and Agreement and such other sublicenses shall remain in full force and effect.

10.8 Effect of Termination on Sublicenses. In the event of termination of Agreement, any sublicense granted by Company under Agreement shall remain in effect and is hereby assigned to Hospital, provided that (i) Company or the Sublicensee provides Hospital with an unredacted copy of such agreement within [**] days after termination of Agreement, unless an unredacted copy previously has been provided to Hospital; (ii) the Sublicensee agrees in writing to an assignment of such sublicense to Hospital and to the payment of all consideration to Hospital that otherwise would have been payable in connection with such sublicense to Hospital by Company under Agreement; (iii) any obligations in such sublicense that are greater than or inconsistent with the obligations of Hospital under Agreement or the nature of Hospital as an academic and non-profit entity shall be reduced in scope to match those in Agreement, if practicable, or terminated if such reduction in scope is not practicable; and (iv) the Sublicensee agrees in writing that all obligations arising prior to such assignment remain the responsibility of

Company and that Hospital is released from any and all liability relating to such obligations; otherwise said sublicense will be terminated.

10.9 Effects of Termination of Agreement. Upon termination of Agreement or any of the licenses hereunder for any reason, final reports in accordance with Section 5 shall be submitted to Hospital and all royalties and other payments, including without limitation any unreimbursed Patent Costs, accrued or due to Hospital as of the termination date shall become immediately payable. Company shall cease, and shall cause its Affiliates and Sublicensees to cease under any sublicense granted by Company, all Sales and uses of Products and Processes upon such termination, subject to Sections 10.8 and 10.10. The termination or expiration of Agreement or any license granted hereunder shall not relieve Company, its Affiliates or its Sublicensees of obligations arising before such termination or expiration.

10.10 Inventory. Upon early termination of Agreement other than for Company default under Section 10.2 or 10.3, Company, Company Affiliates and Company Sublicensees may complete and sell any work-in-progress and inventory of Products that exist as of the effective date of termination provided that (i) Company pays Hospital the applicable running royalty or other amounts due on such Net Sales in accordance with the terms and conditions of Agreement, and (ii) Company, Company Affiliates and Sublicensees shall complete and sell all work-in-progress and inventory of Products within [**] months after the effective date of termination. Upon expiration of Agreement, Company shall pay to Hospital the royalties set forth in Section 4.5(a) on Net Sales of any Product that was in inventory or was a work-in-progress on the date of expiration of the Agreement.

ARTICLE 11. COMPLIANCE WITH LAW

11.1 Compliance. Company shall have the sole obligation for compliance with, and shall ensure that any Affiliates and Sublicensees comply with, all government statutes and regulations that relate to Products and Processes, including, but not limited to, those of the Food and Drug Administration and the Export Administration, as amended, and any applicable laws and regulations of any other country in the License Territory. Company agrees that it shall be solely responsible for obtaining any necessary licenses to export, re-export or import Products or Processes covered by Patent Rights and/or Confidential Information. Company shall indemnify and hold harmless Hospital for any breach of Company's obligations under this Section 11.1.

11.2 Patent Numbers. Company shall cause all Products sold in the United States to be marked with all applicable U.S. Patent Numbers, to the full extent required by United States law. Company shall similarly cause all Products shipped to or sold in any other country to be marked in such a manner as to conform with the patent laws and practices of such country.

ARTICLE 12. MISCELLANEOUS

12.1 Entire Agreement. Agreement constitutes the entire understanding between the Parties with respect to the subject matter hereof and supersedes any prior understandings, whether written, oral or otherwise. **Appendices A, B, C, and D** are hereby incorporated into Agreement.

12.2 Notices. Any notices, reports, waivers, correspondences or other communications required under or pertaining to Agreement shall be in writing and shall be delivered by hand, or sent by a reputable overnight mail service (*e.g.*, Federal Express), or by first class mail (certified or registered), or by facsimile confirmed by one of the foregoing methods, to the other party. Notices will be deemed effective (a) three (3) working days after deposit, postage prepaid, if mailed, (b) the next day if sent by overnight mail, or (c) the same day if sent by facsimile and confirmed as set forth above or delivered by hand. Unless changed in writing in accordance with this Section, the notice address for each Party shall be as follows:

If to Hospital: Executive Director, Innovation
Massachusetts General Hospital
215 First Street
Cambridge, MA 02142
Fax No. [**]

If to Company: Editas Medicine, Inc.
300 Third Street
Cambridge, MA 02142
Attn: CEO
Copy to: Legal Affairs
Fax No. [**]

12.3 Amendment; Waiver. Agreement may be amended and any of its terms or conditions may be waived only by a written instrument executed by an authorized signatory of the Parties or, in the case of a waiver, by the Party waiving compliance. The failure of either Party at any time or times to require performance of any provision hereof shall in no manner affect its rights at a later time to enforce the same. No waiver by either Party of any condition or term shall be deemed as a further or continuing waiver of such condition or term or of any other condition or term.

12.4 Binding Effect. Agreement shall be binding upon and inure to the benefit of and be enforceable by the Parties hereto and their respective permitted successors and assigns.

12.5 Assignment. Company may assign or transfer Agreement: (a) without the consent of Hospital, to an Affiliate of Company or in connection with the transfer or sale of all or substantially all of Company's assets or business related to the Products, Processes and/or Agreement, whether by merger, consolidation, sale of assets, change in control or other transaction, provided that Company promptly shall provide Hospital with a written notice of such assignment including the identity of the assignee or transferee and such assignee or transferee agrees in writing to assume the obligations to Hospital that are being assigned or transferred; and (b) in any other circumstance, only with the prior written consent of Hospital, such consent not to be unreasonably withheld, conditioned or delayed. Company shall notify Hospital in writing of any such assignment and provide a copy of the assignment and assumption agreement or other documentary evidence sufficient to demonstrate Company's compliance with this Section 12.5 within [**] days after such assignment. Failure of an assignee to agree to be bound by the terms hereof or failure of Company to notify Hospital and provide copies of assignment documentation shall be grounds for termination of Agreement for default.

12.6 Force Majeure. Neither Party shall be responsible for delays resulting from causes beyond the reasonable control of such Party, including without limitation fire, explosion, flood, war, sabotage, strike or riot, provided that the nonperforming Party uses commercially reasonable efforts to avoid or remove such causes of nonperformance and continues performance under Agreement with reasonable dispatch whenever such causes are removed.

12.7 Use of Name. Neither Party shall use the name of the other Party or of any trustee, director, officer, staff member, employee, student or agent of the other Party or any adaptation thereof in any advertising, promotional or sales literature, publicity or in any document employed to obtain funds or financing without the prior written approval of the Party or individual whose name is to be used. For Hospital, such approval shall be obtained from Hospital's VP of Public Affairs. This restriction on use of the name of Hospital and any trustee, director, officer, staff member, employee, student or agent of Hospital shall not apply to factual statements identifying Company as a licensee of technology, inventions or intellectual property rights of Hospital (including for this purpose, identifying the past or present affiliation of any consultant, advisor, employee or director of Company).

12.8 Press Release. Notwithstanding the provisions of Section 12.7, after execution of Agreement, the Parties will use reasonable efforts in a timely manner to agree upon a public communications plan that will define the nature and scope of the information relating to Agreement and the relationship among the Parties that will be disclosed publicly and Company may issue a press release in such form as is consistent with such communications plan and mutually acceptable to the Parties. Once such a public statement or public disclosure has been approved in accordance with Sections 12.7 and 12.8, then either Party may appropriately communicate information contained in such permitted statement or disclosure.

12.9 Governing Law. Agreement shall be governed by and construed and interpreted in accordance with the laws of the Commonwealth of Massachusetts, excluding with respect to conflict of laws, except that questions affecting the construction and effect of any patent shall be determined by the law of the country in which the patent shall have been granted. Each Party agrees to submit to the exclusive jurisdiction of the Superior Court for Suffolk County, Massachusetts, and the United States District Court for the District of Massachusetts with respect to any claim, suit or action in law or equity arising in any way out of Agreement or the subject matter hereof.

12.10 Hospital Policies. Company acknowledges that Hospital's employees and medical and professional staff members and the employees and staff members of Hospital's Affiliates are subject to the applicable policies of Hospital and such Affiliates, including, without limitation, policies regarding conflicts of interest, intellectual property and other matters.

12.11 Severability. If any provision(s) of Agreement are or become invalid, are ruled illegal by any court of competent jurisdiction or are deemed unenforceable under then current applicable law from time to time in effect during the term hereof, it is the intention of the Parties that the remainder of Agreement shall not be effected thereby. It is further the intention of the Parties that in lieu of each such provision which is invalid, illegal or unenforceable, there be substituted or added as part of Agreement a provision which shall be as similar as possible in economic and

business objectives as intended by the Parties to such invalid, illegal or enforceable provision, but shall be valid, legal and enforceable.

12.12 Survival. In addition to any specific survival references in Agreement, Articles 1, 11 and 12 and Sections 2.4, 2.5, 4.2, 4.7, 4.8, 5.3, 5.4, 6.4, 6.5, 7.6, 8.1, 8.2, 9.2, 9.3, 10.1 (in the case of expiration of Agreement in accordance with Section 10.1) 10.7, 10.8, 10.9 and 10.10 shall survive termination or expiration of Agreement. Any other rights, responsibilities, obligations, covenants and warranties which by their nature should survive Agreement shall similarly survive and remain in effect.

12.13 Interpretation. The parties hereto are sophisticated, have had the opportunity to consult legal counsel with respect to this transaction and hereby waive any presumptions of any statutory or common law rule relating to the interpretation of contracts against the drafter.

12.14 Headings. All headings are for convenience only and shall not affect the meaning of any provision of Agreement.

12.15 Confidentiality.

(a) Each party agrees to the terms of confidentiality and non-use set forth in **Appendix D**.

(b) All reports and other information disclosed by Company to Hospital pursuant to Sections 2.3, 3, 4 and 5, and any information obtained by Hospital pursuant to any audit conducted under Section 5.4(a), shall be included in the "Confidential Information" (as such term is defined and used in **Appendix D**).

(c) The terms and conditions of Agreement shall be the Confidential Information of each of Company and Hospital.

(d) Notwithstanding anything in **Appendix D** to the contrary, Company may disclose any Confidential Information of Hospital (i) to actual and *bona fide* potential investors, lenders and acquirors/acquirees, merger partners, other financial or commercial partners to the extent necessary in connection with a proposed equity or debt financing of Company, or a proposed acquisition or business combination, or to actual and *bona fide* potential sublicensees, so long as such recipients are bound in writing or by professional obligations to maintain the confidentiality of such information by terms at least as stringent as the terms of Agreement and/or (ii) to the extent required under the rules or regulations of the U.S. Securities and Exchange Commission, or any similar regulatory agency in any country other than the United States, or of any stock exchange, including Nasdaq; provided that, to the extent practicable, Company promptly notifies Hospital prior to such required disclosure, discloses such information only to the extent so required and cooperates reasonably with Hospital's efforts to contest or limit the scope of such disclosure.

IN WITNESS WHEREOF, the Parties have caused Agreement to be executed by their duly authorized representatives as of the Effective Date first written above.

EDITAS MEDICINE, INC.	**GENERAL HOSPITAL CORPORATION**
BY: /s/ Katrine Bosley Name: Katrine Bosley	BY: /s/ Daniel Castro Name: Daniel Castro
TITLE: CEO	TITLE: Director, Business Strategy and Licensing, Innovation Partners Health Care
DATE: 8/2/16	DATE: 8/2/16

Appendix A

DESCRIPTION OF PATENT RIGHTS

MGH Case No.	Status	Filing Date	Application Serial No.
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]

Appendix B

DESCRIPTION OF TECHNOLOGICAL INFORMATION

[**]

Appendix C

SALES REPORTS

AGREEMENT INCOME REPORT — Royalty Income

[MGH][BWH] Agreement # - _____
Licensee - _____
Sub-Licensee - _____

Separate reports must be filed for:

1. ***Each Product sold.***
2. ***Each country of sale, if different deductions or royalty rates apply.***

Product Name: _____
Report Time Period:
From *mm/dd/yyyy* _____
To *mm/dd/yyyy* _____

Country of Sale			
Quantity Sold			
Gross Sales (USD)	$	$	$
Exchange Rate			

Deductions (Itemize)
Please list each deduction separately. Use same definition as appears in Agreement and include the contract paragraph as a reference (Std Section 1.22(a)(ii) line item deductions listed below).

A1.			
A2.			
A3.			
A4.			
B.			
Total Deductions	( )	( )	( )
Net Sales			
Royalty Percentage			
Credits (itemize)	( )	( )	( )
Royalties Due	$	$	$

PLEASE ATTACH DETAIL SALES REPORTS AS REQUIRED

Appendix D

CONFIDENTIALITY TERMS AND CONDITIONS

1. Definition of Confidential Information. "Confidential Information" shall mean any information, including but not limited to data, techniques, protocols or results, or business, financial, commercial or technical information, disclosed by one Party (each, a "Discloser") to the other Party (each, a "Recipient") in connection with Agreement and identified as confidential at the time of disclosure. Hospital's Confidential Information shall also include all information disclosed by Hospital to Company in connection with Patent Rights. Capitalized terms used in this **Appendix D** that are not otherwise defined herein have the meanings ascribed in Agreement to which this **Appendix D** is attached and made a part thereof.

2. Exclusions. "Confidential Information" under Agreement shall not include any information that (i) is or becomes publicly available through no wrongful act of Recipient; (ii) was known by Recipient prior to disclosure by Discloser, as evidenced by tangible records; (iii) becomes known to Recipient after disclosure from a third party having an apparent bona fide right to disclose it; (iv) is independently developed or discovered by Recipient without use of Discloser's Confidential Information, as evidenced by tangible records; or (v) is disclosed to another party by Discloser without restriction on further disclosure. The obligations of confidentiality and non-use set forth in Agreement shall not apply with respect to any information that Recipient is required or advised by legal counsel or auditors to disclose or produce (including the filing of this Agreement with applicable authorities) pursuant to applicable law (including securities laws and regulations), court order or other valid legal process or in order to comply with the rules or regulations of any applicable Trading Market; provided that Recipient, if practicable, promptly notifies Discloser prior to such required disclosure, discloses such information only to the extent so required and cooperates reasonably with Discloser's efforts to contest or limit the scope of such disclosure.

3. Permitted Purpose. Recipient shall have the right to, and agrees that it will, use Discloser's Confidential Information solely for the purposes described in Agreement (the "Purpose"), except as may be otherwise specified in a separate definitive written agreement negotiated and executed between the parties.

4. Restrictions. For the term of Agreement and a period of [**] years thereafter (and indefinitely with respect to any individually identifiable health information disclosed by Hospital to Company, if any), each Recipient agrees that: (i) it will not use Confidential Information of the Disclosing Party for any purpose other than as specified herein, including without limitation for its own benefit or the benefit of any other person or entity; and (ii) it will use reasonable efforts (but no less than the efforts used to protect its own confidential and/or proprietary information of a similar nature) not to disclose such Confidential Information to any other person or entity except as expressly permitted hereunder. Recipient may, however, disclose Discloser's Confidential Information only on a need-to-know basis to its and its Affiliates employees, staff members and agents ("Receiving Individuals") who are directly participating in the Purpose and who are informed of the confidential nature of such information, provided Recipient shall be

responsible for compliance by Receiving Individuals with the terms of Agreement and any breach thereof. Each party further agrees not to use the name of the other party or any of its Affiliates or any of their respective trustees, directors, officers, staff members, employees, students or agents in any advertising, promotional or sales literature, publicity or in any document employed to obtain funds or financing without the prior written approval of the party or individual whose name is to be used, in the case of Hospital such approval to be given by the Public Affairs Department. This Section 4 shall survive termination or expiration of Agreement.

5. Right to Disclose. Discloser represents to Recipient that to the best of its knowledge it has the right to disclose to Recipient all of Discloser's Confidential Information that will be disclosed hereunder.

6. Ownership. All Confidential Information disclosed pursuant to Agreement, including without limitation all written and tangible forms thereof, shall be and remain the property of the Discloser. Upon termination of Agreement, if requested by Discloser, Recipient shall return or destroy at Discloser's discretion all of Discloser's Confidential Information, provided that Recipient shall be entitled to keep one copy of such Confidential Information in a secure location solely for the purpose of determining Recipient's legal obligations hereunder.

7. No License. Nothing in this **Appendix D** shall be construed as granting or conferring, expressly or impliedly, any rights by license or otherwise, under any patent, copyright, or other intellectual property rights owned or controlled by Discloser relating to Confidential Information, except as specifically set forth in Agreement.

8. Remedies. Each party acknowledges that any breach of the terms of Section 12.15 of Agreement, including the terms of this **Appendix D**, by it may cause irreparable harm to the other party and that each party is entitled to seek injunctive relief and any other remedy available at law or in equity.

Appendix E

REPRESENTATIONS AND WARRANTIES; LEGEND

1. Representations and Warranties. The letter that Hospital will issue to Company under Section 4.6(d)(ii) in connection with the issuance of any Subsequent Shares [**], will certify as to the following:

Hospital is acquiring the Subsequent Shares solely for its own account for investment purposes and not with a view to, or for offer or sale in connection with, any distribution of said Subsequent Shares;

Hospital acknowledges that the Subsequent Shares are not registered under the Securities Act, or any state securities laws, and that said Subsequent Shares may not be transferred or sold except pursuant to the registration provisions of the Securities Act or pursuant to an applicable exemption therefrom and subject to state securities laws and regulations, as applicable; and

Hospital has had an opportunity to discuss Company's business, management, financial affairs and the terms and conditions of the offering of the Subsequent Shares with Company's management and have had an opportunity to review Company's facilities. Hospital has such knowledge and experience in financial and business matters that it is capable of evaluating the merits and risks of an investment in the Company. Hospital acknowledges receipt of copies of Company's filings pursuant to the Exchange Act. Hospital represents that it is an accredited investor (as that term is defined in Rule 501 of Regulation D under the Securities Act).

2. Legend. A legend substantially in the following form will be placed on the certificate representing the Subsequent Shares:

> "The shares represented by this certificate have not been registered under the Securities Act of 1933, as amended, and may not be sold, transferred or otherwise disposed of in the absence of an effective registration statement under such Act or an opinion of counsel satisfactory to the corporation to the effect that such registration is not required."

Exhibit 31.1

CERTIFICATIONS

I, Katrine S. Bosley, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Editas Medicine, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

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

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

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

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

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

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

Date: November 9, 2016

By: /s/ Katrine S. Bosley
Katrine S. Bosley
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Andrew A.F. Hack, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Editas Medicine, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

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

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

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

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

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

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

Date: November 9, 2016

By: /s/ Andrew A. F. Hack
Andrew A.F. Hack, M.D., Ph.D.
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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

In connection with this Quarterly Report on Form 10-Q of Editas Medicine, Inc. (the "Company") for the period ended September 30, 2016, 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, as adopted pursuant to (section) 906 of the Sarbanes-Oxley Act of 2002, that to the best of her or his knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 9, 2016

By: /s/ Katrine S. Bosley
Katrine S. Bosley
President and Chief Executive Officer

Date: November 9, 2016

By: /s/ Andrew A.F. Hack
Andrew A.F. Hack, M.D., Ph.D.
Chief Financial Officer