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

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**FORM 10-Q**

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(Mark One)

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

For the quarterly period ended June 30, 2019

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

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

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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)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	EDIT	The Nasdaq Stock Market LLC

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

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

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

Large accelerated filer	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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

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

The number of shares of Common Stock outstanding as of August 1, 2019 was 49,515,328.

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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)**

	June 30, 2019	December 31, 2018
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 210,605	\$ 134,776
Marketable securities	107,325	234,179
Accounts receivable	12	30
Prepaid expenses and other current assets	6,298	5,791
Total current assets	<u>324,240</u>	<u>374,776</u>
Property and equipment, net	8,530	40,232
Right-of-use asset	17,477	—
Restricted cash and other non-current assets	5,378	5,378
Total assets	<u>\$ 355,625</u>	<u>\$ 420,386</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 3,981	\$ 5,327
Accrued expenses	8,169	12,813
Deferred revenue, current	56,036	15,712
Operating lease liability	3,648	—
Other current liabilities	1,033	2,048
Total current liabilities	<u>72,867</u>	<u>35,900</u>
Operating lease liability, net of current portion	13,784	—
Deferred revenue, net of current portion	75,911	115,614
Construction financing lease obligation, net of current portion	—	32,417
Other non-current liabilities	1	293
Total liabilities	<u>162,563</u>	<u>184,224</u>
Commitments and contingencies (see note 7)		
Stockholders' equity		
Preferred stock, \$0.0001 par value per share: 5,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, \$0.0001 par value per share: 195,000,000 shares authorized; 49,432,039 and 49,028,907 shares issued, and 49,241,093 and 48,758,951 shares outstanding at June 30, 2019 and December 31, 2018, respectively	5	5
Additional paid-in capital	671,522	652,464
Accumulated other comprehensive income (loss)	45	(29)
Accumulated deficit	<u>(478,510)</u>	<u>(416,278)</u>
Total stockholders' equity	<u>193,062</u>	<u>236,162</u>
Total liabilities and stockholders' equity	<u>\$ 355,625</u>	<u>\$ 420,386</u>

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

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

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2019	2018	2019	2018
Collaboration and other research and development revenues	\$ 2,330	\$ 7,372	\$ 4,399	\$ 11,299
Operating expenses:				
Research and development	23,565	32,718	39,408	54,017
General and administrative	14,414	14,311	31,903	28,498
Total operating expenses	37,979	47,029	71,311	82,515
Operating loss	(35,649)	(39,657)	(66,912)	(71,216)
Other income, net:				
Other (expense) income, net	(68)	154	(111)	336
Interest income, net	1,931	780	3,988	1,219
Total other income, net	1,863	934	3,877	1,555
Net loss	\$ (33,786)	\$ (38,723)	\$ (63,035)	\$ (69,661)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.69)	\$ (0.82)	\$ (1.29)	\$ (1.50)
Weighted-average common shares outstanding, basic and diluted	49,070,574	46,952,059	48,955,043	46,474,685

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

**Editas Medicine, Inc.**  
**Condensed Consolidated Statements of Comprehensive Loss**  
**(unaudited)**  
**(amounts in thousands)**

	<u>Three Months Ended</u> <u>June 30,</u>		<u>Six Months Ended</u> <u>June 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
Net loss	\$ (33,786)	\$ (38,723)	\$ (63,035)	\$ (69,661)
Other comprehensive income (loss):				
Unrealized gain on marketable debt securities	16	53	74	77
Comprehensive loss	<u>\$ (33,770)</u>	<u>\$ (38,670)</u>	<u>\$ (62,961)</u>	<u>\$ (69,584)</u>

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

**Editas Medicine, Inc.**  
**Condensed Consolidated Statements of Stockholders' Equity**  
**(unaudited)**  
**(amounts in thousands)**

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Other Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	<b>Balance at December 31, 2018</b>	48,758,951	\$ 5	\$652,464	\$ (29)	\$ (416,278)
Cumulative effect adjustment for adoption of new accounting guidance	—	—	—	—	803	803
Exercise of stock options	146,171	—	1,533	—	—	1,533
Vesting of restricted common stock awards	18,000	—	410	—	—	410
Stock-based compensation expense	—	—	7,445	—	—	7,445
Unrealized gain on marketable securities	—	—	—	58	—	58
Net loss	—	—	—	—	(29,249)	(29,249)
<b>Balance at March 31, 2019</b>	<u>48,923,122</u>	<u>\$ 5</u>	<u>\$661,852</u>	<u>\$ 29</u>	<u>\$ (444,724)</u>	<u>\$ 217,162</u>
Exercise of stock options	277,259	—	2,894	—	—	2,894
Vesting of restricted common stock units and awards	24,486	—	410	—	—	410
Purchase of common stock under benefit plan	16,226	—	283	—	—	283
Stock-based compensation expenses	—	—	6,083	—	—	6,083
Unrealized gain on marketable securities	—	—	—	16	—	16
Net loss	—	—	—	—	(33,786)	(33,786)
<b>Balance at June 30, 2019</b>	<u>49,241,093</u>	<u>\$ 5</u>	<u>\$671,522</u>	<u>\$ 45</u>	<u>\$ (478,510)</u>	<u>\$ 193,062</u>

  

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Other Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	<b>Balance at December 31, 2017</b>	44,507,960	\$ 4	\$514,002	\$ (76)	\$ (305,850)
Cumulative effect adjustment for adoption of new accounting guidance	—	—	—	—	(474)	(474)
Issuance of common stock from public offering, net of issuance costs of \$0.1 million	1,429,205	—	48,493	—	—	48,493
Issuance of common stock for repayment of notes payable	305,909	—	9,530	—	—	9,530
Issuance of common stock for asset purchase agreement	56,099	—	1,942	—	—	1,942
Exercise of stock options	305,408	—	4,328	—	—	4,328
Vesting of restricted common stock	107,114	—	652	—	—	652
Stock-based compensation expense	—	—	5,878	—	—	5,878
Unrealized gain on marketable securities	—	—	—	24	—	24
Net loss	—	—	—	—	(30,939)	(30,939)
<b>Balance at March 31, 2018</b>	<u>46,711,695</u>	<u>\$ 4</u>	<u>\$584,825</u>	<u>\$ (52)</u>	<u>\$ (337,263)</u>	<u>\$ 247,514</u>
Issuance of common stock for repayment of notes payable	330,617	1	12,500	—	—	12,501
Exercise of stock options	192,687	—	2,597	—	—	2,597
Vesting of restricted common stock	103,481	—	677	—	—	677
Purchase of common stock under benefit plan	14,273	—	362	—	—	362
Stock-based compensation expense	—	—	6,349	—	—	6,349
Unrealized gain on marketable securities	—	—	—	53	—	53
Net loss	—	—	—	—	(38,723)	(38,723)
<b>Balance at June 30, 2018</b>	<u>47,352,753</u>	<u>\$ 5</u>	<u>\$607,310</u>	<u>\$ 1</u>	<u>\$ (375,986)</u>	<u>\$ 231,330</u>

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

	Six Months Ended	
	June 30,	
	2019	2018
<b>Cash flow from operating activities</b>		
Net loss	\$ (63,035)	\$ (69,661)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	14,348	13,552
Depreciation	1,321	1,535
Non-cash research and development expense	—	14,442
Non-cash investment in equity securities	—	(3,667)
Other non-cash items, net	(1,959)	(1,098)
Changes in operating assets and liabilities:		
Accounts receivable	18	317
Prepaid expenses and other current assets	301	(1,414)
Right-of-use asset	1,984	—
Other non-current assets	—	(92)
Accounts payable	(1,459)	984
Accrued expenses	(4,627)	2,032
Deferred revenue	621	3,877
Operating lease liability	(2,325)	—
Other current and non-current liabilities	3	—
Net cash used in operating activities	<u>(54,809)</u>	<u>(39,193)</u>
<b>Cash flow from investing activities</b>		
Purchases of property and equipment	(2,231)	(2,401)
Proceeds from the sale of equipment	36	5
Purchases of marketable securities	(106,569)	(224,465)
Proceeds from maturities of marketable securities	235,500	196,000
Net cash provided by (used in) investing activities	<u>126,736</u>	<u>(30,861)</u>
<b>Cash flow from financing activities</b>		
Proceeds from offering of common stock, net of issuance costs	—	48,471
Proceeds from exercise of stock options	3,619	6,925
Issuances of common stock under benefit plans	283	362
Payments on construction financing lease obligation	—	(410)
Net cash provided by financing activities	<u>3,902</u>	<u>55,348</u>
Net increase (decrease) in cash and cash equivalents	75,829	(14,706)
Cash, cash equivalents and restricted cash, beginning of period	136,395	148,249
Cash, cash equivalents and restricted cash, end of period	<u>\$ 212,224</u>	<u>\$ 133,543</u>
<b>Supplemental disclosure of cash and non-cash activities:</b>		
Fixed asset additions included in accounts payable and accrued expenses	\$ 96	\$ 371
Receivable of proceeds from exercise of stock options	808	—
Cash paid in connection with operating lease liabilities	3,089	—
Right-of-use assets obtained in exchange of operating lease obligations	19,461	—
Reclassification of liability for common stock subject to repurchase	—	4
Issuance of common stock for settlement of liabilities (see note 7)	—	22,030
Issuance of common stock for asset acquisition	—	1,942
Adjustment to deferred revenue for revenue adoption	—	474
Offering costs included in accounts payable and accrued expenses	—	22

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 leading, clinical stage genome editing company dedicated to developing potentially transformative genomic medicines to treat a broad range of serious diseases. 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. The Company has primarily financed its operations through various equity financings, payments received under a research collaboration with Juno Therapeutics, Inc., a Celgene company that is a wholly-owned subsidiary of Celgene Corporation (“Juno Therapeutics”), and payments received under a strategic alliance and option agreement with Allergan Pharmaceuticals International Limited (“Allergan”).

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.

***Liquidity***

As of June 30, 2019, the Company has raised an aggregate of \$328.3 million in net proceeds through the sale of shares of its common stock in public offerings. The Company has incurred annual net operating losses in every year since its inception. The Company expects that its existing cash, cash equivalents and marketable securities at June 30, 2019 and anticipated interest income will enable it to fund its operating expenses and capital expenditure requirements for at least the next 24 months following the date of this Quarterly Report on Form 10-Q. The Company had an accumulated deficit of \$478.5 million at June 30, 2019 and will require substantial additional capital to fund its operations. The Company has never generated any product revenue. 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 generally accepted accounting principles in the United States of America (“GAAP”) 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, 2018 (the “Annual Report”).

The unaudited condensed consolidated financial statements include the accounts of Editas Medicine, Inc. and its wholly owned subsidiary, Editas Securities Corporation. 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 June 30, 2019 and 2018 are referred to as the second quarter of 2019 and 2018, 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.

### *Summary of Significant Accounting Policies*

The Company’s significant accounting policies are described in Note 2, “Summary of significant accounting policies,” to the consolidated financial statements included in the Annual Report. There have been no material changes to the significant accounting policies previously disclosed in the Annual Report other than as noted below.

### *Recent Accounting Pronouncements –Adopted*

#### *Leases*

Effective January 1, 2019, the Company adopted Accounting Standards Update (“ASU”) No. 2016-02, *Leases* (Topic 842) (“ASC 842”), which amends a number of aspects of lease accounting and requires entities to recognize right-of-use assets and liabilities on the balance sheet.

At the inception of an arrangement the Company determines whether the arrangement contains a lease. If a lease is identified in an arrangement, the Company recognizes a right-of-use asset and liability on its balance sheet and determines whether the lease should be classified as a finance or operating lease. The Company does not recognize assets or liabilities for leases with lease terms of less than 12 months. Lease payments for short-term leases are recorded to operating expense on a straight-line basis over the lease term and variable lease payments are recorded in the period in which the obligation for those payments is incurred.

A lease qualifies as a finance lease if any of the following criteria are met at the inception of the lease: (i) there is a transfer of ownership of the leased asset to the Company by the end of the lease term, (ii) the Company holds an option to purchase the leased asset that it is reasonably certain to exercise, (iii) the lease term is for a major part of the remaining economic life of the leased asset, (iv) the present value of the sum of lease payments equals or exceeds substantially all of the fair value of the leased asset, and (v) the nature of the leased asset is specialized to the point that it is expected to provide the lessor no alternative use at the end of the lease term. All other leases are recorded as operating leases.

Finance and operating lease assets and liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term using the discount rate implicit in the lease. If the rate is not readily determinable, the Company utilizes its incremental borrowing rate at the lease commencement date. Operating lease assets are further adjusted for prepaid or accrued lease payments. Operating lease payments are expensed using the

straight-line method as an operating expense over the lease term. Finance lease assets are amortized to depreciation expense using the straight-line method over the shorter of the useful life of the related asset or the lease term. Finance lease payments are bifurcated into (i) a portion that is recorded as imputed interest expense and (ii) a portion that reduces the finance liability associated with the lease.

The Company does not separate lease and non-lease components when determining which lease payments to include in the calculation of its lease assets and liabilities. Variable lease payments are expensed as incurred. If a lease includes an option to extend or terminate the lease, the Company reflects the option in the lease term if it is reasonably certain it will exercise the option.

The Company elected the modified-retrospective transition method, pursuant to which the Company recognized a cumulative-effect adjustment of \$0.8 million to the opening balance of accumulated deficit on January 1, 2019 associated with de-recognizing the net asset balance recorded in property and equipment, net and the offsetting construction financing lease liability related to the Company's headquarters which was previously accounted for under the built-to-suit guidance in Accounting Standards Codification ("ASC") 840, *Leases* ("ASC 840"). This resulted in a reversal of \$32.6 million from total assets and \$33.4 million from total liabilities. All prior period balances are presented in accordance with ASC 840. As of January 1, 2019, the Company recorded a right-of-use asset of \$19.5 million and lease liability of \$19.7 million associated with the adoption of ASC 842. In addition, the Company elected to adopt the package of three practical expedients for leases that commenced prior to January 1, 2019, allowing it not to reassess (i) whether any expired or existing contracts contain leases, (ii) the lease classification for any expired or existing leases and (iii) the initial indirect costs for any existing leases. The Company did not elect the hindsight practical expedient which allows the Company to reassess the lease term as it was not relevant to the Company's leases.

As of June 30, 2019, the Company had only operating leases and has recorded the \$17.5 million asset balance and \$17.4 million liability balance in right-of-use assets and operating lease liabilities, respectively, in the condensed consolidated balance sheet as of June 30, 2019. The Company has finalized changes to its controls to support lease accounting and related disclosures under the new standard.

#### *Stock-Based Compensation*

Effective January 1, 2019, the Company adopted ASU No. 2018-07, *Compensation – Stock Compensation: Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07"), which simplified the accounting for share-based payments to non-employees by aligning it with the accounting for share-based payments to employees, with certain exceptions. The new guidance expands the scope of ASC 718, *Compensation – Stock Compensation* ("ASC 718"), which supersedes the guidance in ASC 505-50, *Equity-Based Payments to Non-Employees* ("ASC 505-50"). In accordance with the new guidance, the Company will account for share-based payments to non-employees by recognizing stock-based compensation expense equal to the grant date fair value of the share-based payment ratably over the requisite service period. The Company estimates the grant date fair value for each stock option using the Black-Scholes option-pricing model. For restricted stock awards and restricted stock unit awards, the Company estimates the value of each award using intrinsic value, which is based on the value of the underlying common stock less any purchase price. On the date of adoption, the Company estimated the fair value for all unvested non-employee stock options and restricted shares. The unvested stock-based compensation will be recorded over the remaining requisite service period.

#### ***Recent Accounting Pronouncements – Issued But Not Yet Adopted***

In August 2018, the FASB issued ASU No. 2018-13, *Disclosure Framework - Changes to the Disclosure Requirements for Fair Value Measurement* ("ASU 2018-13"), which modifies certain disclosure requirements on fair value measurements. The amendments regarding changes in unrealized gains and losses, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements and the narrative description of measurement uncertainty are required to be applied prospectively for only the most recent interim or annual period presented in the initial fiscal year of adoption. All other amendments are required to be applied retrospectively to all periods presented upon their effective date. ASU 2018-13 is effective for fiscal years beginning after December 15, 2019 and interim periods within those years. The Company does not anticipate a material impact to disclosures as a result of the adoption of ASU 2018-13.

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In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* (“ASU 2016-13”). The standard requires that a financial asset or a group of financial assets measured at amortized cost basis to be presented at the net amount expected to be collected. Under current GAAP, a company only considered past events and current conditions in measuring an incurred loss. Under ASU 2016-13, the information that a company must consider is broadened in developing an expected credit loss estimate for assets measured either collectively or individually. The use of forecasted information incorporates more timely information in the estimate of expected credit loss. The new guidance will be effective for annual and interim periods beginning after December 15, 2019. Early adoption is permitted for annual and interim periods beginning after December 15, 2018. The guidance is applied using a modified retrospective, or prospective approach, depending on a specific amendment. The Company does not anticipate a material impact to disclosures as a result of the adoption of ASU 2016-13.

### 3. Cash Equivalents, Marketable Securities and Equity Securities

Cash equivalents, marketable securities and equity securities consisted of the following at June 30, 2019 (in thousands):

June 30, 2019	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash equivalents and marketable securities:				
Money market funds	\$ 210,605	\$ —	\$ —	\$ 210,605
U.S. Treasuries	59,851	20	—	59,871
Government agency securities	47,429	25	—	47,454
Equity securities included in other non-current assets:				
Corporate equity securities	3,667	—	—	3,667
<b>Total</b>	<b>\$ 321,552</b>	<b>\$ 45</b>	<b>\$ —</b>	<b>\$ 321,597</b>

Cash equivalents, marketable securities and equity securities consisted of the following at December 31, 2018 (in thousands):

December 31, 2018	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash equivalents and marketable securities:				
Money market funds	\$ 130,049	\$ —	\$ —	\$ 130,049
U.S. Treasuries	208,754	—	(24)	208,730
Government agency securities	29,940	—	(5)	29,935
Equity securities included in other non-current assets:				
Corporate equity securities	3,667	—	—	3,667
<b>Total</b>	<b>\$ 372,410</b>	<b>\$ —</b>	<b>\$ (29)</b>	<b>\$ 372,381</b>

At June 30, 2019, the Company held two securities that were in an unrealized loss position. The aggregate fair value of securities held by the Company in an unrealized loss position for less than 12 months at June 30, 2019 was \$9.0 million, and there were no securities held by the Company in an unrealized loss position for more than 12 months. Pursuant to the adoption of ASU No. 2016-01, *Recognition and Measurement of Financial Assets and Financial Liabilities*, the Company records changes in the fair value of its investments in corporate equity securities to “Other (expense) income, net” in the Company’s condensed consolidated statements of operations. The Company records unrealized gains (losses) on available-for-sale debt securities as a component of accumulated other comprehensive income (loss) until such gains and losses are realized.

As of June 30, 2019, the Company did not intend to sell, and was not more likely than not required to sell, the

debt securities in an unrealized loss position before recovery of their amortized cost bases. Furthermore, the Company has determined that there were no material changes in the credit risk of the debt securities. As a result, the Company determined it did not hold any marketable securities with any other-than-temporary impairment as of June 30, 2019.

There were no realized gains or losses on available-for-sale securities during the six months ended June 30, 2019 or 2018.

#### 4. Fair Value Measurements

Assets measured at fair value on a recurring basis as of June 30, 2019 were as follows (in thousands):

<b>Financial Assets</b>	<b>June 30, 2019</b>	<b>Quoted Prices in Active Markets for Identical Assets (Level 1)</b>	<b>Significant Other Observable Inputs (Level 2)</b>	<b>Significant Unobservable Inputs (Level 3)</b>
<b>Cash equivalents:</b>				
Money market funds	\$ 210,605	\$ 210,605	\$ —	\$ —
<b>Marketable securities:</b>				
U.S. Treasuries	59,871	59,871	—	—
Government agency securities	47,454	47,454	—	—
<b>Restricted cash and other non-current assets:</b>				
Corporate equity securities	3,667	—	3,667	—
Money market funds	1,619	1,619	—	—
<b>Total financial assets</b>	<b>\$ 323,216</b>	<b>\$ 319,549</b>	<b>\$ 3,667</b>	<b>\$ —</b>

Assets measured at fair value on a recurring basis as of December 31, 2018 were as follows (in thousands):

<b>Financial Assets</b>	<b>December 31, 2018</b>	<b>Quoted Prices in Active Markets for Identical Assets (Level 1)</b>	<b>Significant Other Observable Inputs (Level 2)</b>	<b>Significant Unobservable Inputs (Level 3)</b>
<b>Cash equivalents:</b>				
Money market funds	\$ 130,049	\$ 130,049	\$ —	\$ —
U.S. Treasuries	4,487	4,487	—	—
<b>Marketable securities:</b>				
U.S. Treasuries	204,243	204,243	—	—
Government agency securities	29,935	29,935	—	—
<b>Restricted cash and other non-current assets:</b>				
Corporate equity securities	3,667	—	3,667	—
Money market funds	1,619	1,619	—	—
<b>Total financial assets</b>	<b>\$ 374,000</b>	<b>\$ 370,333</b>	<b>\$ 3,667</b>	<b>\$ —</b>

There were no transfers between fair value measurement levels during the six months ended June 30, 2019.

**5. Accrued Expenses**

Accrued expenses consisted of the following (in thousands):

	As of	
	June 30, 2019	December 31, 2018
Intellectual property and patent related fees	\$ 2,952	\$ 1,939
Employee related expenses	2,727	5,201
Professional service expenses	1,026	475
Process and platform development expenses	700	1,044
Sublicensing and success payment expenses	412	3,750
Other expenses	352	404
Total accrued expenses	<u>\$ 8,169</u>	<u>\$ 12,813</u>

## 6. Property and Equipment, net

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

	As of	
	June 30, 2019	December 31, 2018
Laboratory equipment	\$ 12,501	\$ 10,892
Computer equipment	733	733
Construction-in-progress	456	—
Leasehold improvements	289	289
Furniture and office equipment	166	166
Software	118	118
Building	—	35,167
Total property and equipment	14,263	47,365
Less: accumulated depreciation	(5,733)	(7,133)
Property and equipment, net	\$ 8,530	\$ 40,232

For additional information related to the removal of the building asset, refer to Footnote 7.

## 7. Commitments and Contingencies

### Leases

In 2016, the Company entered into a lease agreement for 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 and continues 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 payments commenced in November 2016 and continue through the term of the lease and are subject to increases over the term of the lease. The Company subleased approximately 10,000 square feet of the Hurley Street premises pursuant to a sublease, which commenced in February 2017 and terminated in June 2018.

In accordance with ASC 840 and 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 construction financing lease obligation, on its balance sheet for the total amount of the project costs incurred whether funded by the Company or the landlord. 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 would remain on the Company’s balance sheet at its historical cost, and such asset would be depreciated over its estimated useful life of 30 years. The Company bifurcated its future lease payments pursuant to the lease into (i) a portion that was allocated to the building and (ii) a portion that was allocated to the land on which the building is located, which was recorded as rental expense. Although the Company did not begin making lease payments pursuant to the lease until November 2016, the portion of the lease obligation allocated to the land was treated for accounting purposes as an operating lease that commenced upon execution of the lease in February 2016.

Effective January 1, 2019, the Company adopted ASC 842 and derecognized the balances relating to the building, accumulated depreciation and the corresponding construction financing lease as summarized in the table below (in thousands). In applying the ASC 842 transition guidance, the Company determined that the lease should be classified as an operating lease and recorded a right-of-use asset and lease liability on the effective date, accordingly.

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	As of January 1, 2019
Property and equipment, net	\$ 32,627
Other current liabilities	\$ (1,014)
Construction financing lease obligation, net of current portion	\$ (32,417)
Accumulated deficit	\$ 803

The Company has two other operating leases for laboratory space. One of those leases, which commenced in April 2017, was amended in April 2018 and continues until March 2021. The second lease commenced in January 2018 and continues until June 2021. Base rent payments commenced at the beginning of each lease term and continues through the term of the respective lease. Base rent is also subject to increases over the term of the lease. In prior periods, the Company accounted for these leases as operating leases under ASC 840 and recognized straight-line rent expense over the remaining non-cancellable lease terms. As part of its adoption of ASC 842, effective January 1, 2019, the Company elected to apply the package of practical expedients which, among other things, allowed the Company to carry forward its existing lease classification under ASC 840. Additionally, the Company recorded right-of-use assets and lease liabilities for these operating leases on the effective date.

The Company's leases are included on its condensed consolidated balance sheet as follows (in thousands):

	As of	
	June 30, 2019	January 1, 2019
Right-of-use asset	\$ 17,477	\$ 19,461
Lease liability, current	\$ (3,648)	\$ (3,848)
Lease liability, noncurrent	\$ (13,784)	\$ (15,909)

The following table contains a summary of the operating lease costs recognized under ASC 842 and other information pertaining to the Company's operating leases during the three and six months ended June 30, 2019 (in thousands):

	Three Months Ended June 30, 2019	Six Months Ended June 30, 2019
Operating lease costs	\$ 1,421	\$ 2,842
Variable lease costs	\$ 261	\$ 528
Total lease costs	<u>\$ 1,682</u>	<u>\$ 3,370</u>

Maturities of the Company's lease liabilities in accordance with ASC 842 as of June 30, 2019 were as follows (in thousands):

	Six Months Ended June 30, 2019
<b>Maturity of lease liabilities:</b>	
2019	\$ 2,293
2020	\$ 5,620
2021	\$ 4,761
2022	\$ 4,470
2023	\$ 3,802
Thereafter	\$ —
Total minimum lease payments	\$ 20,946
Less: imputed interest	\$ (3,514)
Total operating lease liabilities at June 30, 2019	<u>\$ 17,432</u>

The above table excludes \$0.6 million of legally binding minimum lease payments for leases executed but not yet commenced as of June 30, 2019. Commencement occurred in July 2019.

The weighted-average remaining lease terms are 4.0 years and the weighted-average discount rate is 9.27%.

#### ***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, and other license agreements between the Company and Broad. 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. During the three and six months ended June 30, 2019, the Company recognized \$3.5 million and \$6.9 million in expense for such reimbursement, respectively. During the three and six months ended June 30, 2018, the Company recognized \$3.5 million and \$8.0 million in expense for such reimbursement, respectively.

#### ***Success Payments***

In 2016, the Company entered into patent license agreements with each of The General Hospital Corporation, d/b/a Massachusetts General Hospital (“MGH”), and Broad (collectively, the “2016 License Agreements”). Pursuant to the terms of the 2016 License Agreements, the Company is required to make certain success payments to MGH, Broad and Wageningen University (“Wageningen” and such payments, collectively, the “Success Payments”), payable in cash or, at the Company’s election, common stock in the case of MGH or, in the case of Broad and Wageningen, promissory notes payable in cash or, at the Company’s election subject to certain conditions, common stock of the Company. The Success Payments are payable, if and when, the Company’s market capitalization reaches specified thresholds for a specific period of time or upon a sale of the Company for consideration in excess of those thresholds (collectively, the “Payment Conditions”).

The Success Payments were historically accounted for under the provisions of ASC 505-50. Effective January 1, 2019, the Company adopted ASU 2018-07, which expands the scope of ASC 718 and supersedes ASC 505-50. In accordance with ASC 718, the Company will recognize a Success Payment when it becomes probable that the Payment Conditions will be met. However, the Company has the right to terminate any of the 2016 License Agreements at will upon written notice. Absent any of the Payment Conditions being achieved prior to termination, the Company would not be obligated to pay any Success Payments. As such, the Company will recognize the expense and liability associated with each Success Payment when it is probable that the amounts will become due. The Company records this expense as a research and development expense in its condensed consolidated statements of operations.

#### ***Research Funding Payments***

In June 2018, the Company entered into a sponsored research agreement (the “Sponsored Research Agreement”) with Broad. Pursuant to the terms of the Sponsored Research Agreement, the Company is required to make certain research funding payments to Broad, payable by promissory note, cash or common stock. Under the Sponsored Research Agreement, the Company is obligated to make payments of research funding to Broad in the event the Company’s market capitalization reaches specified thresholds ranging from a mid-nine digit dollar amount to a low-eleven digit dollar amount (“Market Cap Research Funding”) or a Company sale for consideration ranging from a mid-nine digit dollar amount to a low-eleven digit dollar amount (“Company Sale Research Funding” and, collectively with the Market Cap Research Funding, the “Research Funding Payments”). In connection with entering into the Sponsored Research Agreement, the Company confirmed that the first two Research Funding Payments of \$5.0 million and \$7.5 million were due and payable to Broad (the “Initial Research Payments”).

Other than the Initial Research Payments, the Company is not required to make additional Research Funding Payments if the Company, whether directly or through its affiliates or sublicensees, is not researching, developing, or commercializing products based on or incorporating inventions developed under the Sponsored Research Agreement and exclusively licensed to the Company from Broad or based on or incorporating CRISPR technology owned, co-owned, or



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controlled by Broad and otherwise licensed to the Company, subject to certain exclusions. The Research Funding Payments were historically accounted for under the provisions of ASC Topic 505-50. Effective January 1, 2019, the Company adopted ASU 2018-07, which expands the scope of ASC 718 and superseded ASC 505-50. Under ASC 718, the Company will recognize the expenses and liability associated with each Research Funding Payment when it is probable that the amounts will become due. The Company records this expense as a research and development expense in its statements of operations.

In June 2018, in connection with the Company's entry into the Sponsored Research Agreement and the trigger of the Initial Research Payments, the Company issued promissory notes in an aggregate principal amount of \$12.5 million to Broad (the "Initial Research Notes") bearing interest at a rate of 4.8% per annum, except with respect to \$7.5 million of the principal, which would not start accruing interest until November 2018. The Company fully settled the outstanding principal and accrued interest on the Initial Research Notes by issuing 330,617 shares of common stock to Broad in June 2018.

***Litigation***

The Company is not a party to any litigation and did not have contingency reserves established for any litigation liabilities as of June 30, 2019 or December 31, 2018.

**8. Collaboration and License Agreements**

The Company has entered into multiple collaboration, license and strategic agreements with third parties that typically involve the Company granting licenses to the Company's intellectual property, receiving licenses to intellectual property rights and/or performing research and development services in exchange for upfront fees, milestone payments and royalty payments to or from the Company. The terms and conditions as well as the accounting analysis for the Company's significant collaboration, license and strategic agreements are described in Note 9, "Significant Agreements," to the consolidated financial statements included in the Annual Report. There have been no material changes to the terms and conditions, or the accounting conclusions previously disclosed in the Annual Report.

***Collaboration Revenue***

As of June 30, 2019, the Company's contract liabilities were primarily related to the Company's collaboration with Juno Therapeutics and strategic alliance with Allergan. The following table presents changes in the Company's accounts receivable and contract liabilities for the six months ended June 30, 2019 (in thousands):

	Balance at December 31, 2018	Additions	Deductions	Balance at June 30, 2019
<b>For the six months ended June 30, 2019</b>				
Accounts receivable	\$ 30	\$ 12	\$ (30)	\$ 12
Contract liabilities:				
Deferred revenue	\$ 131,326	\$ 5,000	\$ (4,379)	\$ 131,947

During the three and six months ended June 30, 2019, the Company recognized the following collaboration revenue (in thousands):

	Three Months Ended	Six Months Ended
	June 30, 2019	
<b>Revenue recognized in the period from:</b>		
Amounts included in deferred revenue at the beginning of the period	\$ 2,318	\$ 4,379
Performance obligations satisfied in previous periods	\$ —	\$ —

***Juno Therapeutics Collaboration Agreement***

In May 2015, the Company entered into a Collaboration and License Agreement with Juno Therapeutics and in May 2018 the Company and Juno Therapeutics entered into an Amended and Restated Collaboration and License Agreement (collectively, the "Juno Agreement").

There have been no material changes to the transaction price in the three or six months ended June 30, 2019. As of June 30, 2019, the only milestones that were included in the transaction price were milestones that had been contractually earned and received. The outstanding milestones were fully constrained, as a result of the uncertainty regarding whether any of the milestones will be achieved. As of June 30, 2019, Juno Therapeutics has not exercised any of its options associated with the material rights identified in the Juno Agreement and no revenue has been recognized related to the material rights. Amounts allocated to each of the material rights will be recognized when the material right has been exercised or when the respective option has lapsed.

During the three and six months ended June 30, 2019, the Company did not recognize revenue related to the Juno Agreement. During the three and six months ended June 30, 2018, the Company recognized \$3.5 million and \$4.5 million in revenue related to the Juno Agreement. As of June 30, 2019 and December 31, 2018, there was approximately \$32.0 million of deferred revenue related to the Juno Agreement, of which \$32.0 million and \$2.8 million were classified as short-term, respectively, in the accompanying condensed consolidated balance sheets. In addition, there were no amounts classified in accounts receivable on the condensed balance sheets related to the Juno Agreement.

#### ***Allergan Pharmaceuticals Strategic Alliance and Option Agreement and Profit-Sharing Agreement***

In March 2017, the Company entered into a Strategic Alliance and Option Agreement with Allergan to discover, develop, and commercialize new gene editing medicines for a range of ocular disorders (the “Allergan Agreement”). Over a seven-year research term, Allergan will have an exclusive option to exclusively license from the Company up to five collaboration development programs for the treatment of ocular disorders (each a “CDP”), including the Company’s Leber congenital amaurosis 10 (“LCA10”) program and the related experimental therapeutic EDIT-101 to treat LCA10 (the “LCA10 Program”).

There have been no material changes to the transaction price in the three or six months ended June 30, 2019. As of June 30, 2019, the only milestone that was included in the transaction price had been contractually earned and received. The outstanding milestones were fully constrained, as a result of the uncertainty regarding whether any of the milestones will be achieved. The Company has concluded that the options to purchase five development and commercialization licenses are considered a marketing offer as the options did not provide any discounts or other rights that would be considered a material right in the arrangement. In August 2018, Allergan exercised its right to the LCA10 Program and the option fee was recognized as revenue during the three months ended September 30, 2018 when the control transferred to Allergan.

During the three and six months ended June 30, 2019, the Company recognized revenue related to the Allergan Agreement of approximately \$2.3 million and \$4.3 million, respectively. During the three and six months ended June 30, 2018, the Company recognized revenue related to the Allergan Agreement of approximately \$2.9 million. As of June 30, 2019 and December 31, 2018, there was \$94.9 million and \$99.2 million of deferred revenue related to the Allergan Agreement, respectively, of which \$72.4 million and \$86.4 million is classified as long-term on the condensed consolidated balance sheet, respectively. There were no amounts classified in accounts receivable on the condensed balance sheets related to the Allergan Agreement.

The Company and an affiliate of Allergan (together with Allergan, the “Allergan Entities”) agreed to equally split U.S. profits and losses for the LCA10 Program in the United States and to co-develop the LCA10 Program in the United States (the “Profit-Sharing Agreement”). The Company accounts for the Profit-Sharing Arrangement with respect to the LCA10 Program within the scope of ASC Topic 808, *Collaborative Arrangements*, given that the Company and the Allergan Entities are active participants in future research and development activities and all parties are exposed to significant risks and rewards dependent on the commercial success of such activities. During the three and six months ended June 30, 2019, the Company and the Allergan Entities incurred \$3.7 million and \$9.0 million in expense associated with the LCA10 Program, respectively, of which the Company recognized 50% in operating expense during such periods.

#### **9. Stock-based Compensation**

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Research and development	\$ 3,853	\$ 3,996	\$ 7,235	\$ 7,906
General and administrative	2,640	3,028	7,113	5,646
Total stock-based compensation expense	<u>\$ 6,493</u>	<u>\$ 7,024</u>	<u>\$ 14,348</u>	<u>\$ 13,552</u>

***Restricted Stock and Restricted Stock Unit Awards***

Restricted stock awards are subject to repurchase rights. Accordingly, the Company has recorded the proceeds from the issuance of restricted stock awards as a liability in its condensed consolidated balance sheets. The restricted stock liability is reclassified into stockholders' equity as the restricted stock vests.

The following table summarizes restricted stock and restricted stock unit awards activity as of December 31, 2018 and June 30, 2019:

	Shares	Weighted Average Grant Date Fair Value Per Share
<b>Unvested restricted stock and restricted stock unit awards as of December 31, 2018</b>	270,000	\$ 28.05
Issued	400,722	\$ 21.52
Vested	(42,486)	\$ 27.09
Forfeited	(36,763)	\$ 21.33
<b>Unvested restricted stock and restricted stock unit awards as of June 30, 2019</b>	<u>591,473</u>	\$ 24.11

As of June 30, 2019, the Company had \$10.7 million in unrecognized stock-based compensation expense related to unvested restricted stock and unvested restricted stock unit awards.

***Stock Options***

The following is a summary of stock option activity for the six months ended June 30, 2019:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
<b>Outstanding at December 31, 2018</b>	4,689,786	\$ 23.80	7.9	\$ 20,686
Granted	653,707	\$ 21.55		
Exercised	(423,430)	\$ 10.46		
Cancelled	(467,185)	\$ 27.84		
<b>Outstanding at June 30, 2019</b>	<u>4,452,878</u>	\$ 24.31	7.8	\$ 19,365
Exercisable at June 30, 2019	<u>2,220,677</u>	\$ 21.21	6.9	\$ 15,108

As of June 30, 2019, the Company had unrecognized stock-based compensation expense related to its employee and director stock options of \$32.9 million, which the Company expects to recognize over the remaining weighted average vesting period of 2.41 years. As of June 30, 2019, the Company had unrecognized stock-based compensation expense related to its non-employee stock options of \$1.1 million, which the Company expects to recognize over the remaining weighted average vesting period of 0.74 years.

**10. 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 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.

In connection with at-the-market offerings consummated by the Company during 2018, the Company sold 2,536,205 shares of its common stock. The issuance of these shares resulted in a significant increase in the Company's weighted-average shares outstanding and is expected to continue to impact the year-over-year comparability of the Company's net loss per share calculations for the remainder of 2019.

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 June 30,	
	2019	2018
Unvested restricted stock and restricted stock unit awards	591,473	306,000
Outstanding stock options	4,452,878	5,101,679
Total	<u>5,044,351</u>	<u>5,407,679</u>

## 11. Related-Party Transactions

The Company received \$0.4 million in rent and facility-related fees from a related party during the six months ended June 30, 2018 in connection with the Company's Hurley Street sublease and no rent or facility-related payments were received from this related party during the six months ended June 30, 2019.

## 12. Subsequent Events

On August 6, 2019, the Company's Board of Directors appointed Cynthia Collins as President and Chief Executive Officer of the Company, effective August 19, 2019. Ms. Collins previously served as the Company's interim President and Chief Executive Officer under a consulting agreement. The Company will grant stock options with both time-based and performance-based vesting and a restricted stock unit award subject to time-based vesting pursuant to the terms of the offer letter entered into by the Company and Ms. Collins.

## 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, 2018, which was filed with the Securities and Exchange Commission ("SEC") on March 1, 2019 (the "2018 Annual Report").*

*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, clinical stage genome editing company dedicated to developing potentially transformative genomic medicines to treat a broad range of serious diseases. We have developed a proprietary genome editing platform based on CRISPR technology and we continue to expand its capabilities. Our product development strategy is to target genetically addressable diseases where gene editing can be used to enable or enhance therapeutic outcomes for patients. Genetically addressable diseases include genetically defined diseases that may be treated by correcting a disease-causing gene and genetically treatable diseases that do not necessarily have a single, disease causing gene, but which nonetheless may be treated by editing the genome to ameliorate or eliminate the signs or symptoms of the disease. We are advancing both *in vivo* CRISPR medicines, in which the medicine is injected or infused into the patient to edit the cells inside their body, and engineered cell medicines, in which cells are edited with our technology and then administered to the patient. While our discovery efforts have ranged across several different genetically addressable diseases and therapeutic areas, the two areas where our programs are more mature are ocular diseases and engineered cell medicines to treat blood diseases and cancer.

In ocular diseases, our most advanced program is designed to address a specific genetic form of retinal degeneration called Leber congenital amaurosis 10 (“LCA10”), a disease for which we are not aware of any available therapies and only one other potential treatment in clinical trials in the United States and Europe. In October 2018, we filed an investigational new drug application (“IND”) for a Phase 1/2 clinical trial for EDIT-101 (also known as AGN-151587), an experimental medicine to treat LCA10, which was accepted by the United States Food and Drug Administration (“FDA”) in November 2018. We and our partner Allergan Pharmaceuticals International Limited (together with its affiliate, “Allergan”) recently began patient screening and aim to begin patient dosing in the second half of 2019, enrolling approximately 18 patients in the United States and Europe.

As part of our long term strategy, we have developed and articulated goals for our pipeline of experimental medicines and our company that we are working to achieve by the end of 2022. These goals, which we call “EM22,” include having at least three experimental medicines in early stage clinical trials and at least two additional experimental medicines in or ready for late stage clinical trials. In addition, we aim to have a pipeline characterized by potential best-in-class medicines and to be a company with the leading genome editing platform and organizational culture.

In May 2015, we entered into a collaboration with Juno Therapeutics, Inc., a Celgene company that is a wholly-owned subsidiary of Celgene Corporation (“Juno Therapeutics”), a leader in the emerging field of immuno-oncology, to develop novel engineered T cell therapies for cancer, which was amended and restated in May 2018. In March 2017, we entered into a strategic alliance and option agreement with Allergan to discover, develop, and commercialize new gene editing medicines for a range of ocular disorders. In July 2018, Allergan exercised its option to develop and commercialize EDIT-101 and paid us \$15.0 million in connection with such exercise (the “EDIT-101 Option Exercise Payment”). We and Allergan subsequently entered into a co-development and commercialization agreement under which we will co-develop and equally split profits and losses for EDIT-101 in the United States. In December 2018, we also received a \$25.0 million payment from Allergan in connection with the acceptance of the IND for EDIT-101 (the “EDIT-101 Milestone Payment”).

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. Except for EDIT-101, all of our research programs are still in the preclinical or research stage of development and the risk of failure of all of our research programs 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 (the “IPO”), follow-on public offerings of our common stock including through at-the-market offerings, private placements of our preferred stock, payments received under our collaboration with Juno Therapeutics and payments received under our strategic alliance and co-development and commercialization agreements with Allergan and its affiliate. From inception through June 30, 2019, we raised an aggregate of \$682.3 million to fund our operations.

Since inception, we have incurred significant operating losses. Our net losses were \$63.0 million and \$69.7 million for the six months ended June 30, 2019 and 2018, respectively. As of June 30, 2019, we had an accumulated deficit of \$478.5 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; progress the clinical development of EDIT-101 with Allergan; seek to identify additional research programs and additional product candidates; initiate preclinical testing and clinical trials for other 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, 2019 or the foreseeable future.

## **Financial Operations Overview**

### ***Revenue***

To date, we have not generated any revenue from product sales and we 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 each of May 2016 and July 2017, we received a milestone payment of \$2.5 million. In May 2018, in connection with the amendment and restatement of our collaboration agreement with Juno Therapeutics to expand our collaboration to add an additional research program, we received \$5.0 million for amending the agreement and two \$2.5 million milestone payments for technical progress in a research program. In addition, we were entitled to receive up to \$22.0 million in research support over the five years of the collaboration and across the four programs under the collaboration, subject to adjustment in accordance with the terms of the agreement and, as of June 30, 2019, we had recognized an aggregate of \$17.7 million of such research support. During the six months ended June 30, 2019, we did not recognize any research support from Juno Therapeutics. As of June 30, 2019, we recorded \$32.0 million of deferred revenue, all of which is classified as current on our condensed consolidated balance sheet, related to the collaboration.

In connection with entering into our strategic alliance with Allergan, we received an upfront payment of \$90.0 million from Allergan and in 2019 we received \$15.0 million for the EDIT-101 Option Exercise Payment and \$25.0 million for the EDIT-101 Milestone Payment. Through June 30, 2019, we had recognized an aggregate of \$35.1 million in revenue related to our strategic alliance with Allergan, which includes all of the EDIT-101 Option Exercise Payment and a portion of the EDIT-101 Milestone Payment. During the six months ended June 30, 2019, we recognized \$4.3 million in revenue related to our strategic alliance with Allergan. As of June 30, 2019, we recorded \$94.9 million of deferred revenue, \$72.5 million of which is classified as long-term on the condensed consolidated balance sheet, related to the upfront payment from Allergan and a portion of the EDIT-101 Milestone Payment. For additional information about our revenue recognition policy related to the Juno Therapeutics collaboration or the Allergan agreement, see “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Estimates—Revenue Recognition” in the Annual Report.

For the foreseeable future, we expect substantially all of our revenue will be generated from our collaboration with Juno Therapeutics, our strategic alliance with Allergan, any other collaborations or agreements we may enter into and anticipated interest income.

***Expenses***

*Research and Development Expenses*

Research and development expenses consist primarily of costs incurred for our research and development 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 acquiring and maintaining licenses under our third-party licensing agreements, including any sublicensing or success payments made to our licensors.

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, IND-enabling studies and natural history 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 a product, if and when approved, whether alone or in collaboration with others;
- acceptance of a 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. As a result of Allergan's exercise of its option to license EDIT-101 and our entry into a profit-sharing arrangement with Allergan in the United States for EDIT-101, our obligations to fund such program in the United States will represent 50% of the total costs related to developing and commercializing the program in the United States.

We do not track research and development costs on a program-by-program basis except for reimbursable amounts that relate to third-party agreements.

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 progress the clinical development of EDIT-101 with Allergan as well as supporting preclinical studies for 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, investor relations, business development, legal, corporate affairs, information technology, facilities and human resource functions. Other significant costs include corporate facility costs not otherwise included in research and development expenses, legal fees related to intellectual property 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 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 expenses associated with operating as 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 intellectual property related expenses specifically, given the ongoing nature of the opposition and interference 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"), we anticipate general and administrative expenses will continue to be significant. Some of our in-licensed patents and patent applications under our license agreement with Broad and Harvard are subject to priority disputes, and we anticipate that our obligation to reimburse Broad and Harvard for expenses related to these disputes during future periods will be substantial until such proceedings are resolved.

#### *Other Income (Expense), Net*

For the six months ended June 30, 2019, other income (expense), net consisted primarily of interest income and accretion of discounts associated with marketable securities, partially offset by loss on disposal of property and equipment.

For the six months ended June 30, 2018, other income (expense), net consisted primarily of interest income, accretion of discounts associated with marketable securities, and rental income from our former subtenant, partially offset by interest expense on our construction financing lease obligation.

#### **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.

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 2018 Annual Report.

**Results of Operations**

***Comparison of the Three Months ended June 30, 2019 and 2018***

The following table summarizes our results of operations for the three months ended June 30, 2019 and 2018, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Three Months Ended June 30,		Dollar Change	Percentage Change
	2019	2018		
Collaboration and other research and development revenues	\$ 2,330	\$ 7,372	\$ (5,042)	(68) %
Operating expenses:				
Research and development	23,565	32,718	(9,153)	(28) %
General and administrative	14,414	14,311	103	(1) %
Total operating expenses	<u>37,979</u>	<u>47,029</u>	<u>(9,050)</u>	<u>(19) %</u>
Other income, net:				
Other (expense) income, net	(68)	154	(222)	n/m
Interest income, net	1,931	780	1,151	n/m
Total other income, net	<u>1,863</u>	<u>934</u>	<u>929</u>	<u>99 %</u>
Net loss	<u>\$ (33,786)</u>	<u>\$ (38,723)</u>	<u>\$ 4,937</u>	<u>(13) %</u>

For our results of operations, we have included the respective percentage of changes, unless greater than 100% or less than (100)%, in which case we have denoted such changes as not meaningful (n/m).

*Collaboration and other research and development revenues*

Collaboration and other research and development revenues decreased by \$5.0 million, to \$2.3 million for the three months ended June 30, 2019 from \$7.4 million for three months ended June 30, 2018. This decrease was primarily attributable to a \$3.5 million decrease in revenue recognized pursuant to our collaboration agreement with Juno Therapeutics and \$3.9 million in revenue recognized for an upfront payment in connection with an out-license arrangement entered into during the second quarter of 2018, partially offset by a \$2.3 million increase in revenue recognized pursuant to our strategic alliance with Allergan.

*Research and development expenses*

Research and development expenses decreased by \$9.2 million, to \$23.6 million for the three months ended June 30, 2019 from \$32.7 million for the three months ended June 30, 2018. The following table summarizes our

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research and development expenses for the three months ended June 30, 2019 and 2018, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Three Months Ended June 30,		Dollar Change	Percentage Change
	2019	2018		
Process and platform development expenses	\$ 10,623	\$ 6,889	\$ 3,734	54 %
Employee related expenses	5,513	4,941	572	12 %
Stock-based compensation expenses	3,853	3,996	(143)	(4) %
Facility expenses	2,149	1,486	663	45 %
Other expenses	1,015	729	286	39 %
Sublicensing and success payment expenses	412	14,677	(14,265)	(97) %
Total research and development expenses	<u>\$ 23,565</u>	<u>\$ 32,718</u>	<u>\$ (9,153)</u>	<u>(28) %</u>

The decrease in research and development expenses for the three months ended June 30, 2019 compared to the three months ended June 30, 2018 was primarily attributable to:

- approximately \$14.3 million in decreased sublicensing and success payment expenses primarily relating to the \$12.5 million notes payable that were issued to Broad and settled in shares of our common stock during the second quarter of 2018 and \$2.2 million in sublicense fees owed to our licensors in connection with milestone payments received from Juno Therapeutics and upfront consideration received from a licensee in connection with an out-license, partially offset by \$0.4 million in accrued sublicensing expenses related to an upfront payment received in connection with an out-license that we entered into during the second quarter of 2019; and
- approximately \$0.1 million in decreased stock-based compensation expense.

These decreases were partially offset by approximately \$3.7 million in increased process and platform development expense, mostly relating to a license arrangement that was entered into during the second quarter of 2019, approximately \$0.7 million in increased facility related expenses due to expanding lab and manufacturing space, approximately \$0.6 million in increased employee related expenses due to an increase in the size of our workforce, and approximately \$0.3 million in increased other expenses.

*General and administrative expenses*

General and administrative expenses increased by \$0.1 million, to \$14.4 million for the three months ended June 30, 2019 from \$14.3 million for the three months ended June 30, 2018. The following table summarizes our general and administrative expenses for the three months ended June 30, 2019 and 2018, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Three Months Ended June 30,		Dollar Change	Percentage Change
	2019	2018		
Intellectual property and patent related fees	\$ 4,352	\$ 5,426	\$ (1,074)	(20) %
Professional service expenses	3,435	1,961	1,474	75 %
Employee related expenses	2,760	2,842	(82)	(3) %
Stock-based compensation expenses	2,640	3,028	(388)	(13) %
Other expenses	1,227	1,054	173	16 %
Total general and administrative expenses	<u>\$ 14,414</u>	<u>\$ 14,311</u>	<u>\$ 103</u>	<u>1 %</u>

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The increase in general and administrative expenses for the three months ended June 30, 2019 compared to the three months ended June 30, 2018 was primarily attributable to:

- approximately \$1.5 million in increased professional service expenses due to an increase in our use of professional service providers; and
- approximately \$0.2 million in increased other expenses including facility related expenses.

These increases were partially offset by \$1.1 million in decreased intellectual property and patent related fees, approximately \$0.4 million in decreased stock-based compensation expenses, and approximately \$0.1 million in decreased employee related expenses.

*Other income, net*

For the three months ended June 30, 2019, other income, net was \$1.9 million, which was primarily attributable to interest income and accretion of discounts associated with marketable securities, partially offset by a loss on disposal of property and equipment.

For the three months ended June 30, 2018, other income, net was \$0.9 million, which was primarily attributable to interest income, accretion of discounts associated with marketable securities, and rental income from our former subtenant, partially offset by interest expense on our former construction financing lease obligation.

***Comparison of the Six Months ended June 30, 2019 and 2018***

The following table summarizes our results of operations for the six months ended June 30, 2019 and 2018, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Six Months Ended June 30,		Dollar Change	Percentage Change
	2019	2018		
Collaboration and other research and development revenues	\$ 4,399	\$ 11,299	\$ (6,900)	(61) %
Operating expenses:				
Research and development	39,408	54,017	(14,609)	(27) %
General and administrative	31,903	28,498	3,405	11 %
Total operating expenses	<u>71,311</u>	<u>82,515</u>	<u>(11,204)</u>	(14) %
Other income, net:				
Other (expense) income, net	(111)	336	(447)	n/m
Interest income, net	3,988	1,219	2,769	n/m
Total other income, net	<u>3,877</u>	<u>1,555</u>	<u>2,322</u>	n/m
Net loss	<u>\$ (63,035)</u>	<u>\$ (69,661)</u>	<u>\$ 6,626</u>	(10) %

*Collaboration and other research and development revenues*

Collaboration and other research and development revenues decreased by \$6.9 million, to \$4.4 million for the six months ended June 30, 2019 from \$11.3 million for six months ended June 30, 2018. This decrease was primarily attributable to a \$4.5 million decrease in revenue recognized pursuant to our collaboration agreement with Juno Therapeutics and \$3.9 million in revenue recognized for an upfront payment in connection with an out-license arrangement entered into during the second quarter of 2018, partially offset by a \$1.4 million increase in revenue recognized in the second quarter of 2019 pursuant to our strategic alliance with Allergan.

*Research and development expenses*

Research and development expenses decreased by \$14.6 million, to \$39.4 million for the six months ended June 30, 2019 from \$54.0 million for the six months ended June 30, 2018. The following table summarizes our research and

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development expenses for the six months ended June 30, 2019 and June 30, 2018, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Six Months Ended June 30,		Dollar Change	Percentage Change
	2019	2018		
Process and platform development expenses	\$ 15,017	\$ 17,128	\$ (2,111)	(12) %
Employee related expenses	10,697	9,807	890	9 %
Stock-based compensation expenses	7,235	7,906	(671)	(8) %
Facility expenses	3,870	2,880	990	34 %
Other expenses	2,177	1,619	558	34 %
Sublicensing and success payment expenses	412	14,677	(14,265)	(97) %
Total research and development expenses	<u>\$ 39,408</u>	<u>\$ 54,017</u>	<u>\$ (14,609)</u>	<u>(27) %</u>

The decrease in research and development expenses for the six months ended June 30, 2019 compared to the six months ended June 30, 2018 was primarily attributable to:

- approximately \$14.3 million in decreased sublicensing and success payment expenses primarily relating to the \$12.5 million notes payable that were issued to Broad and settled in shares of our common stock during the second quarter of 2018 and \$2.2 million in sublicense fees owed to our licensors in connection with milestone payments received from Juno Therapeutics and upfront consideration received from a licensee in connection with an out-license, partially offset by \$0.4 million in accrued sublicensing expenses related to an upfront payment received in connection with an out-license that we entered into during the second quarter of 2019;
- approximately \$2.1 million in decreased process and platform development expenses, mostly due to the expense of certain non-capitalized intangible assets during the first quarter of 2018, partially offset by a license arrangement that was entered into during the second quarter of 2019; and
- approximately \$0.7 million in decreased stock-based compensation expense mostly due to a decrease in non-employee stock option expense.

These decreases were partially offset by approximately \$1.0 million in increased facility related expenses, approximately \$0.9 million in increased employee related expenses due to an increase in the size of our workforce, and approximately \$0.6 million in increased other expenses.

*General and administrative expenses*

General and administrative expenses increased by \$3.4 million, to \$31.9 million for the six months ended June 30, 2019 from \$28.5 million for the six months ended June 30, 2018. The following table summarizes our general and administrative expenses for the six months ended June 30, 2019 and 2018, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Six Months Ended June 30,		Dollar Change	Percentage Change
	2019	2018		
Intellectual property and patent related fees	\$ 9,343	\$ 11,832	\$ (2,489)	(21) %
Stock-based compensation expenses	7,113	5,646	1,467	26 %
Professional service expenses	6,829	3,255	3,574	n/m
Employee related expenses	6,260	5,619	641	11 %
Other expenses	2,358	2,146	212	10 %
Total general and administrative expenses	<u>\$ 31,903</u>	<u>\$ 28,498</u>	<u>\$ 3,405</u>	<u>12 %</u>

The increase in general and administrative expenses for the six months ended June 30, 2019 compared to the six months ended June 30, 2018 was primarily attributable to:

- approximately \$3.6 million in increased professional service expenses;
- approximately \$1.5 million in increased stock-based compensation expenses due to an increase in employee stock option expense;
- approximately \$0.6 million in increased employee related expenses; and
- approximately \$0.2 million in increased other expenses including facility related expenses.

These increases were partially offset by approximately \$2.5 million in decreased intellectual property and patent related fees.

*Other income (expense), net*

For the six months ended June 30, 2019, other income, net was \$3.9 million, which was primarily attributable to interest income and accretion of discounts associated with marketable securities, partially offset by a loss on disposal of property and equipment.

For the six months ended June 30, 2018, other income, net was \$1.6 million, which was primarily attributable to interest income, accretion of discounts associated with marketable securities, and rental income from our former subtenant, partially offset by interest expense on our former construction financing lease obligation.

**Liquidity and Capital Resources**

***Sources of Liquidity***

From inception through June 30, 2019, we funded our operations primarily through proceeds from private placements of our preferred stock of \$163.3 million, net proceeds of \$328.3 million from our public offerings of our common stock, and payments from Allergan and Juno Therapeutics. As of June 30, 2019, we had cash, cash equivalents and marketable securities of \$317.9 million.

In addition to our existing cash, cash equivalents and marketable securities we are eligible to earn milestone payments and are entitled to cost reimbursement under our collaboration agreement with Juno Therapeutics. Additionally, under our strategic alliance with Allergan, we are eligible to earn milestone payments, certain reimbursement for EDIT-101 costs in the United States and certain option exercise or extension payments. Our ability to earn and the timing of the milestone payments are dependent upon the timing and outcome of our development, regulatory and commercial activities and, as such, are uncertain at this time. As of June 30, 2019, our right to contingent payments under our collaboration agreement with Juno Therapeutics and our strategic alliance with Allergan are our only significant committed potential external sources of funds.

[Table of Contents](#)*Cash Flows*

The following table provides information regarding our cash flows for the six months ended June 30, 2019 and 2018 (in thousands):

	Six Months Ended June 30,	
	2019	2018
Net cash provided by (used in):		
Operating activities	\$ (54,809)	\$ (39,193)
Investing activities	126,736	(30,861)
Financing activities	3,902	55,348
Net increase (decrease) in cash and cash equivalents	<u>\$ 75,829</u>	<u>\$ (14,706)</u>

*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.

During the six months ended June 30, 2019, we received an upfront payment related to an out-license arrangement which increased deferred revenue. This amount was offset by operating expenses that relate to our on-going preclinical and clinical activities, patent costs and license fees, costs related to obtaining additional research facilities and increased costs as a result of staffing needs due to our expanding operations.

During the six months ended June 30, 2018, we received a \$10.0 million payment related to the amended and restated collaboration agreement with Juno Therapeutics, which was partially recognized during the second quarter of 2018, resulting in a net increase of deferred revenue of \$3.9 million. This amount was offset by operating expenses that related to our on-going preclinical activities, sublicensing and success payments, patent costs, and increased employee related expenses due to an increase in the size of our workforce.

*Net Cash Provided by Investing Activities*

The cash provided by investing activities for the six months ended June 30, 2019 was primarily the result of higher proceeds from maturities of marketable securities than purchases of marketable securities that were partially offset by \$2.2 million in equipment purchases.

The cash used in investing activities for the six months ended June 30, 2018 was primarily the result of \$2.4 million in equipment purchases as well as an increase in purchases of marketable securities that were partially offset by maturities of marketable securities.

*Net Cash Provided by Financing Activities*

The cash provided by financing activities for the six months ended June 30, 2019 was primarily related to \$3.6 million in proceeds for exercises of options for our common stock and \$0.3 million from the issuance of our common stock under our equity plans.

The cash provided by financing activities for the six months ended June 30, 2018 was primarily related to \$48.5 million in proceeds received from public offerings of common stock, \$6.9 million in proceeds for exercises of options for our common stock and \$0.4 million from the issuance of our common stock under our equity plans, partially offset by payments on the construction financing obligation of \$0.4 million.

*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; progress the clinical development of

EDIT-101 with Allergan; seek to identify product candidates and additional research programs; initiate preclinical testing and clinical trials for other product candidates we identify and develop; maintain, expand, and protect our intellectual property portfolio, including reimbursing our licensors for 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, since 2016 we have incurred, and in future years we expect to continue to incur, significant 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, cash equivalents, and marketable securities at June 30, 2019 and anticipated interest income will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months following the date of this Quarterly Report on Form 10-Q. 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 and our strategic alliance with Allergan;
- 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);
- whether Allergan exercises any additional options under our strategic alliance;
- 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 June 30, 2019, 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 Annual Report and in our Quarterly Report on Form 10-Q for the quarter ending March 31, 2019, which was filed with the Securities and Exchange Commission on May 8, 2019.

#### **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 SEC rules.

#### **Effects of Inflation**

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 six months ended June 30, 2019 or 2018.

### **Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

We are exposed to market risk related to changes in interest rates. As of June 30, 2019, we had cash and cash equivalents of \$210.6 million, primarily held in money market mutual funds, and marketable securities of \$107.3 million, primarily 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 June 30, 2019 were denominated in the United States dollar and we believe that we do not have any material exposure to foreign currency exchange rate risk.



**Item 4. Controls and Procedures.**

**Evaluation of Disclosure Controls and Procedures**

Our management, with the participation of our Chief Executive Officer and interim Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2019. 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 SEC’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 June 30, 2019, our Chief Executive Officer and interim 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**

We regularly review our system of internal control over financial reporting to ensure we maintain an effective internal control environment. We continue to create new processes and controls as well as improve our existing environment to increase efficiencies. Improvements may include such activities as implementing new, more efficient systems, and consolidating activities. During the first quarter of 2019, we implemented certain internal controls in connection with our adoption of Accounting Standards Update No. 2016-02, *Leases* (Topic 842). There were no other changes in our internal control over financial reporting that occurred during the period covered by this Quarterly Report on Form 10-Q that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II. OTHER INFORMATION

### Item 1. Legal Proceedings.

From time to time, we may become involved in litigation or other legal proceedings relating to claims arising from the ordinary course of business. There can be no assurance that any proceedings that result from these third-party actions will be resolved in our favor. In addition, if they are not resolved in our favor, there can be no assurance that the result will not have a material adverse effect on our business, financial condition, results of operations, or prospects. Certain of our intellectual property rights, including ones licensed to us under our licensing agreements, are subject to, and from time to time may be subject to, priority and validity disputes. For additional information regarding these matters, see “Item 1A. Risk Factors—Risks Related to Our Intellectual Property.” Regardless of outcome, litigation or other legal proceedings can have an adverse impact on us because of defense and settlement costs, diversion of management resources, and other factors.

### 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 \$110.0 million, \$120.3 million, \$97.2 million, and \$72.9 million for the years ended December 31, 2018, 2017, 2016 and 2015, respectively. As of June 30, 2019, we had an accumulated deficit of \$478.5 million. We have financed our operations primarily through public offerings of our common stock, private placements of our preferred stock, our collaboration with Juno Therapeutics, Inc., a Celgene company that is a wholly-owned subsidiary of Celgene Corporation (“Juno Therapeutics”), and payments under our strategic alliance with Allergan Pharmaceuticals International Limited (together with its affiliate, “Allergan”). We have devoted substantially 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;
- commence enrollment in and progress the clinical development with Allergan of EDIT-101 to treat Leber congenital amaurosis (“LCA”) 10 (“LCA10”);
- 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;

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- 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
- continue to operate as a public company.

We have only recently initiated clinical development with Allergan of EDIT-101 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. Other than EDIT-101, 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. 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, cash equivalents, and marketable securities at June 30, 2019 and anticipated interest income will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months following the date of this Quarterly Report on Form 10-Q. 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 or natural history study trials for the product candidates we may develop;
- the costs of progressing the clinical development with Allergan of EDIT-101 to treat LCA10;
- 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 and our strategic alliance with Allergan;
- 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);
- whether Allergan exercises any additional options under our strategic alliance;
- 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 significant committed external source of funds, other than our right to payments under our collaboration agreement with Juno Therapeutics, 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, undertaking preclinical studies and preparing to undertake clinical trials. Except for EDIT-101 to treat LCA10, all of our research programs are still in the preclinical or research stage of development, and their risk of failure of all of our research programs is high. We have not yet demonstrated an ability to successfully initiate or 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 collaboration 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;
- 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 arrangements;
- 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 therapeutic 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.*

We have concentrated our research and development efforts on our genome editing platform, which uses CRISPR technology. Our future success depends on the successful development of this novel genome editing therapeutic approach. To date, no therapeutic product that utilizes genome editing, including CRISPR technology, has been approved in the United States or Europe and there have been only a limited number of clinical trials involving the use of a therapeutic utilizing genome editing technologies. Because we have not yet dosed any patients with our product candidates, 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 genomic medicine field, we are aware of a limited number of gene

therapy products that have received marketing authorization from the FDA and the EMA. 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 Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research (“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 committee that reviews and oversees the use of biological agents. 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 allowed its initiation. The same applies in the European Union. The EMA’s Committee for Advanced Therapies (“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 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 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 understanding and 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.

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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, academic scientists in several countries, including the United States, have reported on their attempts to edit the genome of human embryos as part of basic research. In addition, in November 2018, it was reported that Dr. Jiankui He, a Chinese biophysics researcher who was an associate professor in the Department of Biology of the Southern University of Science and Technology in Shenzhen, China, claimed he had created the first human genetically edited babies, twin girls. This claim, and another that Dr. He had helped create a second gene-edited pregnancy, was subsequently confirmed by Chinese authorities and was negatively received by the public, in particular those in the scientific community. In the United States, germline editing for clinical application has been expressly prohibited since enactment of a December 2015 U.S. FDA ban on such activity. Prohibitions are also in place in the United Kingdom, across most of Europe, in China, and many other countries around the world. In the United States, 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. Basic research on embryos is more tightly controlled in many other European countries.

Moreover, in 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. Other than EDIT-101 to treat LCA10, 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 strategic alliance with Allergan, 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 gene editing technology using Cas9 and Cpf1 enzymes, but other genome editing technologies may be discovered that provide significant advantages over CRISPR/Cas9 or CRISPR/Cpf1, which could materially harm our business.***

To date, we have focused our efforts on genome editing technologies using CRISPR and the Cas9 and Cpf1 enzymes. Other companies have previously undertaken research and development of genome editing technologies using zinc finger nucleases, engineered meganucleases, and transcription activator-like effector nucleases, but to date none has obtained marketing approval for a product candidate. There can be no certainty that the CRISPR/Cas9 or CRISPR/Cpf1 technology will lead to the development of genomic medicines, that other genome editing technologies will not be considered better or more attractive for the development of medicines or that either Cas9 or Cpf1, the two CRISPR associated proteins that we use, may be useful or successful in developing therapeutics. For example, Cas9 or Cpf1 may be determined to be less attractive than other CRISPR enzymes, including CRISPR enzymes 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 technology using a Cas9 or Cpf1 enzyme, 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 certain 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. 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 EDIT-101. Except for EDIT-101, 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 and development of EDIT-101 to treat 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 EDIT-101 by Allergan 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 clinical trials for EDIT-101;
- successful completion of preclinical studies and investigational new drug (“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.

The foregoing also applies to our collaborators to the extent we have partnered, sold or licensed any of our research programs to them. For instance, Allergan has exercised its option to license EDIT-101 and, although we have entered into a profit-sharing arrangement to equally split the profits and costs of such program in the United States and we will continue to work with Allergan on the development and commercialization of such program, in the event a dispute arises, Allergan will have final decision making authority. If we or our collaborators 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 (a “BLA”) to the FDA or a marketing authorization application (an “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 or our collaborators will successfully develop or commercialize EDIT-101, or any of our other research programs. If we or any of our collaborators and 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 our proposed delivery modes, combined with CRISPR technology, have a limited history, if any, of being tested clinically. 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 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 a limited history, if any, of being evaluated in human clinical trials. 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 therapeutic 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 (“IECs”) 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 patients 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 IECs 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 patients 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;

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- 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 challenging for the rare genetically defined diseases we are targeting. In addition, if patients are unwilling to participate in our genome 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. For example, ProQR Therapeutics N.V. has already enrolled LCA10 patients in its clinical trial, which may limit the number of potential

patients available to enroll in the ongoing Phase 1/2 clinical study for EDIT-101.

Patient enrollment is also affected by other factors, including:

- severity of the disease under investigation;
- size of the patient population and process for identifying patients;
- 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 patient informed consent;
- risk that enrolled patients 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, EDIT-101 for the treatment of LCA10 has a limited patient pool from which to draw for enrollment in a clinical trial, as 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 affected.

***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 authorities;
- 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 the 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 biotechnology and pharmaceutical industries, including in the gene therapy, genome editing and cell therapy fields, are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on intellectual property and proprietary products. 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. 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 CRISPR technology. Other companies developing CRISPR technology or therapies using CRISPR technology include Arbor Biotechnologies, Caribou Biosciences, Casebia Therapeutics, CRISPR Therapeutics, ERS Genomics, Exonics Therapeutics, Intellia Therapeutics, Locus Biosciences, ToolGen Inc. (“ToolGen”) and TRACR Hematology. In addition, there have been and may continue to be discoveries of new CRISPR-based gene editing technologies. There are additional companies developing therapies using other genome editing technologies, including base editing, transcription activator-like effector nucleases, meganucleases, Mega-TALs, and zinc finger nucleases. These companies include Beam Therapeutics Inc., bluebird bio, Cellectis, Poseida Therapeutics, Precision Biosciences and Sangamo Therapeutics. Additional companies developing gene therapy products include Abeona Therapeutics, Adverum Biotechnologies, AGTC Therapeutics, Audentes Therapeutics, Homology Medicines, Nightstar Therapeutics, REGENXBIO, Sarepta Therapeutics, Solid Biosciences, Spark Therapeutics, uniQure and Voyager Therapeutics. In addition to competition from other genome editing therapies, gene therapies or cell medicine therapies, any products that we may develop may also face competition from other types of therapies, such as small molecule, antibody, protein, oligonucleotide, or ribonucleic acid therapies. For example, ProQR Therapeutics N.V. is conducting a clinical trial for its experimental treatment using antisense oligonucleotide technology for LCA10.

Many of our current or potential competitors, either alone or with their collaboration partners, may 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 for our most advanced programs 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.***

Some of our most advanced programs, including EDIT-101, focus 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 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, EMA or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs.

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, including the ongoing Phase 1/2 clinical trial for EDIT-101, 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 ensure sufficient clinical material for any clinical



trials we may be conducting or are planning to conduct and meet market demand for any products we develop and commercialize. For example, if the contract manufacturing organizations that we have engaged to manufacture EDIT-101 fail to deliver sufficient amounts or fail to timely deliver EDIT-101 due to any of the risks discussed herein, then we and Allergan may not be able to begin patient dosing in the ongoing Phase 1/2 clinical trial for EDIT-101 in the second half of 2019.

#### **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 or for development of certain of our research programs. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates or research programs.*

We anticipate seeking third-party collaborators for the research, development, and commercialization of certain of the product candidates we may develop or for development of certain of our research programs. 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, and, in March 2017, we entered into a strategic alliance with Allergan focused on discovering, developing, and commercializing new gene editing medicines for a range of ocular disorders. 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 and, in the case of our strategic alliance with Allergan, whether they exercise any additional options to commercialize a product. 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, and alliance arrangements we may enter into under which our research programs may be involved and potential product candidates may be developed, including our strategic alliance with Allergan, pose the following risks to us:

- Collaborators may have significant discretion in determining the efforts and resources that they will apply to these collaborations.
- 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.
- 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.

- 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.
- 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, as the case may be. 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 or certain of our research programs, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates or programs.

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 or allies. 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 have a limited ability to manufacture materials for our research programs and preclinical studies and we do not operate any significant manufacturing facilities. We primarily 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 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 and/or collaborators are unable to obtain or maintain patent protection with respect to our CRISPR 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 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 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 or Cpf1. Our owned and in-licensed patents may not cover CRISPR technology in conjunction with a protein other than Cas9 or Cpf1. If our competitors commercialize the CRISPR technology in conjunction with a protein other than Cas9 or Cpf1, 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. Our licensors are currently, and we or our licensors may in the future become, subject to a third party pre-issuance submission of prior art to the United States Patent and Trademark Office (the “USPTO”) or opposition, derivation, revocation, re-examination, post-grant and *inter partes* review, or interference proceedings and other similar 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 or our licensors may need the cooperation of any such co-owners of our owned and in-licensed patents in order to enforce such patents against third parties, and such cooperation may not be provided to us or our licensors. 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 and patent applications 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 U.S. government has certain rights, including 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 example, our licensors, including The Broad Institute, Inc. (“Broad”), have granted the U.S. government non-exclusive, non-transferable, irrevocable, paid-up licenses to practice or have practiced for or on behalf of the United States, the inventions described in certain of our in-licensed patents and patent applications, including certain aspects of our in-licensed CRISPR technology. If the government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. These rights may permit the U.S. 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 U.S. 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 any of the foregoing 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 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 agreements with Broad, and Broad and the President and Fellows of Harvard College (“Harvard”), the licensors may, under certain circumstances, grant a license to the patents that are the subject of such license agreements to a third party. Such third party would have full rights to the patent rights that are the subject of such licenses, which could impact our competitive position and enable a third party to commercialize products similar to our future product candidates and technology. Furthermore, under these license agreements, Broad has the right, after specified periods of time and subject to certain limitations, to designate gene targets for which Broad, whether alone or together with an affiliate or third party, has an interest in researching and developing products that would otherwise be covered by rights licensed to us under the agreements. Any of the foregoing would narrow the scope of our exclusive rights to the patents and patent applications we have in-licensed from Broad. The terms of these license agreements are described more fully under “Part I—Business—Our Collaborations and Licensing Strategy” in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018. In addition, our rights to our in-licensed patents and patent applications are dependent, in part, on inter-institutional or other operating agreements between the joint owners of such in-licensed patents and patent applications. If one or more of such joint owners breaches such inter-institutional or operating agreements, our rights to such in-licensed patents and patent applications may be adversely affected, which could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

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, Harvard, and The General Hospital Corporation, d/b/a Massachusetts General Hospital, 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 to the prosecution, maintenance, enforcement and defense of patents and patent applications that we in-license from them, given the ongoing nature of the interference, opposition and re-examination proceedings involving the patents licensed to us under our license agreement with Harvard and Broad, and given that 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. In connection with these reimbursement obligations, we incurred expenses in aggregate amounts of \$14.2 million, \$18.7 million, \$23.6 million, and \$9.4 million during the years ended December 31, 2018, 2017, 2016, and 2015, respectively, and we incurred expenses in an aggregate amount of \$6.8 million during the six months ended June 30, 2019.

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 and patent applications we in-license. For example, certain patent applications licensed to us by Broad are co-owned with NIH. Broad does not and does not purport to grant any rights in NIH’s interest in these patent applications under our agreement. If other third parties have ownership rights to our in-licensed patents and patent applications, they may be able to license such patents and patent applications 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 and validity disputes. In addition, our owned and in-licensed patents, patent applications and other intellectual property may be subject to further priority and validity disputes, and other similar intellectual property proceedings including inventorship 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.***

Certain 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) and a U.S. patent application (U.S. Serial No. 14/704,551) that are co-owned by Broad and the Massachusetts Institute of Technology (“MIT”), and in some cases Harvard, and in-licensed by us were involved in a first interference with a U.S. patent application (U.S. Serial No. 13/842,859, now U.S. Patent No. 10,266,850) that is co-owned by the University of California, the University of Vienna, and Emmanuelle Charpentier. An interference is a proceeding before the Patent Trial and Appeal Board of the USPTO (“PTAB”) to determine priority of invention of the subject matter of patent claims filed by different parties.

During the preliminary motions phase of the proceeding, the PTAB held that there was no interference-in-fact, meaning that no interference was needed to resolve priority between the parties because the in-licensed claims are directed to subject matter that is patentably distinct from those of the University of California, the University of Vienna, and Emmanuelle Charpentier. The interference proceeding was therefore ended without reaching the priority phase. On appeal, the Court of Appeals for the Federal Circuit (the “CAFC”) affirmed the PTAB’s holding and the University of California, the University of Vienna, and Emmanuelle Charpentier did not appeal to the U.S. Supreme Court for review of this decision. The judgment of no interference-in-fact is therefore final and bars any further interference between the same parties for claims to the same invention that was considered in the interference. The invention that was considered in this first interference was related 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.

As a result, the 12 U.S. patents and one U.S. patent application that we have in-licensed from Broad, acting on behalf of itself, MIT, and Harvard, with respect to which the PTAB had declared an interference were not modified or revoked as a result of this first interference proceeding. However, as discussed below, these 12 U.S. patents and one U.S. patent application are, and may in the future be, subject to further intellectual property proceedings and disputes, including interference proceedings.

On June 24, 2019, the PTAB declared a second interference between 10 pending U.S. patent applications (U.S. Serial No. 15/947,680; 15/947,700; 15/947,718; 15/981,807; 15/981,808; 15/981,809; 16/136,159; 16/136,165; 16/136,168; and 16/136,175) that are owned by the University of California, the University of Vienna, and Emmanuelle Charpentier and the same 12 U.S. patents and one U.S. patent application involved in the first interference that are co-owned by Broad and MIT, and in some cases Harvard, and in-licensed by us. One additional U.S. patent (U.S. Patent No. 9,840,713) that is co-owned by Broad and MIT and in-licensed by us and not involved in the first interference is also included in this second interference. The declaration of interference defined the invention that is subject to the second interference as related to a eukaryotic cell that comprises a target DNA and certain defined CRISPR/Cas9 components including a single-molecule guide RNA that are capable of cleaving or editing the target DNA molecule or modulating transcription of at least one gene encoded thereon.

Although we cannot predict with any certainty how long the second interference 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.



The University of California, the University of Vienna, and Emmanuelle Charpentier or other third parties may file a separate Suggestion of Interference against the Broad patents and patent application that are subject to the interference or other U.S. patents and patent applications that we own or in-license. For example, ToolGen filed Suggestions of Interference in the USPTO on April 13, 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) 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 13 U.S. patents with respect to which the PTAB has declared a second 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.

Our owned and in-licensed patents and patent applications are, or may in the future become, subject to validity disputes in the USPTO and other foreign patent offices. For example, a request for *ex parte* re-examination was filed with the USPTO on February 16, 2016 against one U.S. patent that we have in-licensed from Broad, acting on behalf of itself and MIT (U.S. Patent No. 8,771,945), which is part of the second interference and referenced in the Suggestions of Interference filed by ToolGen. *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 noting that it has jurisdiction over any file that involves a patent involved in an interference. On January 3, 2019, the PTAB lifted the suspension in light of the CAFC's affirmance of the PTAB's no interference-in-fact holding in the first interference. On June 24, 2019, when the PTAB declared the second interference it re-suspended the re-examination. It is uncertain when the PTAB will lift the suspension. If Broad is unsuccessful during the re-examination, U.S. Patent No. 8,771,945 may be revoked or narrowed, which could have a material adverse effect on the scope of our rights under such patent.

The 13 in-licensed U.S. patents and one in-licensed U.S. patent application that are the subject of the second interference (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 13 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 or narrowing in scope of one or more of these in-licensed patents could have a material adverse effect on the conduct of our business, financial condition, results of operations, and prospects.

We or our licensors may also be subject to claims that former employees, collaborators, or other third parties have an interest in our owned or in-licensed patents or patent applications, or other intellectual property rights as an inventor or co-inventor. If we are unable to obtain an exclusive license to any such third party co-owners' interest in such patents, patent applications or other intellectual property rights, 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, including any patents that issue from patent applications, 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, financial condition, results of operations, and prospects.

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. For example, 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 2,931,898 B1) have been revoked in their entirety by the European Patent Office Opposition Division (the "Opposition



Division”). Broad, acting on behalf of itself, MIT and Harvard has filed notices of appeal to the Boards of Appeal of the EPO for review of the Opposition Division’s decisions to revoke these three patents. It is uncertain when or in what manner the Boards of Appeal will act on these appeals. Two other European patents that we have in-licensed from Broad, acting on behalf of itself, MIT and Harvard (European Patent Nos. EP 2,896,697 B1 and EP 2,898,075 B1) were maintained with amended patent claims. The Opposition Division has also initiated opposition proceedings against six other European patents that we have in-licensed from Broad, acting on behalf of itself and MIT, itself, MIT and Harvard, or itself, MIT, Harvard and The Rockefeller University (“Rockefeller”) (European Patent Nos. EP 2,764,103 B1, EP 2,825,654 B1, EP 2,840,140 B1, EP 2,921,557 B1, EP 2,931,892 B1, EP 2,931,897 B1, and EP 3,009,511 B1), and one European patent that we co-own and in-license from Broad, acting on behalf of itself, MIT and The University of Iowa Research Foundation (European Patent No. EP 3,066,201 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. The loss of priority for, or the loss of, these European patents could have a material adverse effect on the conduct of our business. One or more of the third parties that have filed oppositions against these European patents or other third parties may file future oppositions against other European patents that we in-license or own.

If we or our licensors are unsuccessful in any patent related disputes, including interference proceedings, patent oppositions, re-examinations, or other priority, inventorship, or validity disputes to which we or they are subject (including any of the proceedings discussed above), 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 and patent applications. 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 in-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 any interference proceeding or other priority, inventorship, or validity disputes, it could result in substantial costs and be a distraction to our 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. In addition, our intellectual property license agreements may not always include worldwide rights. For example, certain U.S. patent applications licensed to us by Broad include The University of Tokyo (“Tokyo”) and NIH as joint applicants. Broad has only granted a license to us with respect to its interests and to Tokyo’s interests in these U.S. patent applications but not to any foreign equivalents thereof. 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 or licenses 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 and our intellectual property rights 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 genome editing technology, 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, including the amount, if any, that may become due and payable to our licensors in connection with sublicense income. If these events were to occur, they 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 third party patents and patent applications that may be construed to cover our CRISPR technology and product candidates. In order to avoid infringing these third party patents, or patents that issue from these third party patent applications, 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 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 in 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 our CRISPR technology and product candidates we may develop. 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 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 the USPTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. 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 or our collaborators were to initiate legal proceedings against a third party to enforce a patent covering a product candidate we may develop or our technology, including CRISPR genome editing technology, 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, as discussed above, 13 of our in-licensed U.S. patents and one of our in-licensed U.S. patent applications are involved in an interference, and Suggestions of Interference have been filed against certain of our in-licensed U.S. patents, one of these U.S. patents is subject to a re-examination proceeding, opposition proceedings have been initiated against several of our in-licensed European patents and additional interference, re-examination, post-grant review, *inter partes* review, opposition, and other intellectual property proceedings may be initiated in the future. The opposition proceedings have so far resulted in the revocation of three of our in-licensed European patents while maintaining two of our in-licensed European patents with amended claims. In view of certain arguments made by the third parties against the revoked patents and similar arguments made by the third parties against additional other in-licensed European patents under opposition, the opposition proceedings could potentially lead to the revocation of additional in-licensed European patents. These and other proceedings could result in the revocation or 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, 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 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, re-examination, 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 patents and patent applications in this landscape that may be asserted to encompass our CRISPR/Cas9 technology. In particular, we are aware of several separate families of U.S. patents and/or 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 2013/141680 (and its related U.S. Patent No. 9,637,739 and other related U.S. patent applications and foreign counterparts including European Patent No. EP 2,828,386 B1) filed by Vilnius University (which is reported to have exclusively licensed its rights to DuPont Pioneer, which is reported to have licensed certain rights to Caribou Biosciences, which is reported to have non-exclusively licensed certain rights to Intellia Therapeutics and CRISPR Therapeutics), WO 2013/176772 (and its related U.S. Patent No. 10,000,772, 10,113,167, 10,227,611, and 10,266,850, 10,301,651, 10,308,961, 10,337,029, 10,351,878, 10,358,658, and 10,358,659, and other related U.S. patent applications and foreign counterparts including European Patent Nos. EP 2,800,811 B1, EP 3,241,902 B1, and EP 3,401,400 B1 which are being opposed by several parties) 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, ERS Genomics and TRACR Hematology), WO 2014/065596 (and its related U.S. patent applications and foreign counterparts including European Patent No. EP 2,912,175 B1 which is being opposed by several parties) filed by ToolGen, and WO 2014/089290 (and its related U.S. patent applications and foreign counterparts including European Patent Nos. EP 3,138,910 B1, EP 3,138,911 B1, and EP 3,138,912 B1 which are being opposed by several parties) filed by Sigma-Aldrich Co. LLC. 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 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.

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, priority, or validity 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, 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 products that are similar to any product candidates we may develop or utilize similar 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 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.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. On March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty. The United Kingdom had a period of a maximum of two years from the date of its formal notification, which has been extended to October 31, 2019, to negotiate the terms of its withdrawal from, and future relationship with, the European Union. If no formal withdrawal agreement can be reached between the United Kingdom and the European Union, then it is expected that the United Kingdom's membership of the European Union would automatically terminate on the deadline. Discussions between the United Kingdom and the European Union have proven to be extremely difficult to date and will continue to focus on withdrawal issues and transition agreements. However, limited progress to date in these negotiations and the recent appointment of Boris Johnson, who has previously suggested that the country should leave the European Union without an agreement, as the Prime Minister sustains the possibility of the United Kingdom leaving the European Union without a withdrawal agreement and associated transition period in place, which is likely to cause significant market and economic disruption. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, Brexit could materially impact the regulatory regime with respect to the approval of any future product candidate in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product

candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

***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;

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- 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, as further amended by the Health Information Technology for Economic and Clinical Health Act, 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, which 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, which 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 certain state laws that 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 publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization, and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes, or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines, or imprisonment.

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.

***The efforts of the current presidential administration to pursue regulatory reform may limit the FDA's ability to engage in oversight and implementation activities in the normal course, and that could negatively impact our business.***

The current presidential administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. On January 30, 2017, the president issued an executive order, applicable to all executive agencies, including the FDA, that requires that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This executive order includes a budget neutrality provision that requires the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the executive order requires agencies to identify regulations to offset any incremental cost of a new regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within the Office of Management and on February 2, 2017, the administration indicates that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

***Recently enacted and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.***

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, 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 any 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, as amended by the Health Care and Education Affordability Reconciliation Act (the "PPACA"), which became law in 2010, contains provisions of importance to our business, including, without limitation, our ability to commercialize and the prices we may obtain for any of our product candidates and that are approved for sale, the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;

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- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- 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 drug 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. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the ACA, there have been numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by the president on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. According to the Congressional Budget Office, the repeal of the individual mandate will cause 13 million fewer Americans to be insured in 2027 and premiums in insurance markets may rise. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseparable feature of the ACA, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the ACA are invalid as well. The Trump administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the same judge issued an order staying the judgment pending appeal. The Trump administration has represented to the US Court of Appeals for the Fifth Circuit considering this judgment that it does not oppose the lower court's ruling. To that end, on May 1, 2019, the Justice Department filed a brief asking the Court to strike down the entirety of the ACA. Thereafter, on July 10, 2019, the Court of Appeals for the Fifth Circuit heard oral argument in this case. In those arguments, the Trump administration argued in support of upholding the lower court decision. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results

Further, each chamber of the Congress has put forth multiple bills designed to repeal or repeal and replace portions of the ACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the ACA. The Congress will likely consider other legislation to replace elements of the ACA, during the next Congressional session. It is possible that repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in

individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to repeal and replace ACA provisions is highly uncertain in many respects, it is also possible that some of the ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provision.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our potential products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

The current presidential administration has also taken executive actions to undermine or delay implementation of the ACA. Since January 2017, the president has signed two executive orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. One executive order directs federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. The second executive order terminates the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than \$12 billion in ACA risk corridor payments to third-party payors who argued were owed to them. The effects of this gap in reimbursement on third-party payors, the viability of the ACA marketplace, providers, and potentially our business, are not yet known.

The costs of prescription pharmaceuticals has also been the subject of considerable discussion in the United States, and members of Congress and the executive branch have stated that they will address such costs through new legislative and administrative measures. To date, there have been several recent U.S. congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, the executive branch's administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the current presidential administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of



which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

***Fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates.***

If a product candidate is intended for the treatment of a serious or life threatening condition and the product candidate demonstrates the potential to address unmet medical need for this condition, the sponsor may apply for FDA fast track designation. However, a fast track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive fast track designation for our product candidates, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

***Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidates.***

If the FDA determines that a product candidate offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for certain of our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

***We may not be able to obtain orphan drug exclusivity for one or more of our product candidates, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.***

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

In order for the FDA to grant orphan drug exclusivity to one of our products, the agency must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet this standard. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

On August 3, 2017, the Congress passed the FDA Reauthorization Act of 2017 ("FDARA"). FDARA, among other things, codified the FDA's pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the



clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

***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 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.

***Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.***

We are subject to numerous laws and regulations in each jurisdiction outside the United States in which we operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act (“FCPA”) prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain drugs and drug candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

### **Risks Related to Employee Matters, Managing Growth and Information Technology**

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

We are highly dependent on the principal members of our management and scientific teams. Each of these individuals is employed "at will," meaning we or the individual 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. Additionally, although we have an interim Chief Financial Officer, we are actively trying to recruit a candidate to fill this position, as well as the role of Chief Medical Officer, permanently and any inability to fill these position in an expedient manner may have a material adverse effect on our business.

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 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, including a permanent Chief Financial Officer, and a Chief Medical Officer, other 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 have expanded and expect to further expand our development, regulatory, clinical, manufacturing 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, clinical development, manufacturing, and sales and marketing. For example, our total number of employees grew from 55 as of December 31, 2015 to 132 as of December 31, 2018. 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.

***Security breaches and other disruptions to our information technology structure could compromise our information, disrupt our business and expose us to liability, which would cause our business and reputation to suffer.***

In the ordinary course of our business, we collect, process and store sensitive data, including intellectual property, as well as our proprietary business information and that of our suppliers and business partners, employee data, and we may collect personally identifiable information of clinical trial participants when we begin clinical trials. We also rely to a large extent on information technology systems to operate our business, including our financial systems. We have outsourced elements of our confidential information processing and information technology structure, and as a result, we are managing independent vendor relationships with third parties who may or could have access to our confidential information. Similarly, our business partners and other third-party providers possess certain of our sensitive data. The secure maintenance of this information is important to our operations and business strategy. Despite our security measures, our information technology infrastructure (and those of our partners, vendors and third-party providers) may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. We, our partners, vendors, and other third-party providers could be susceptible to third party attacks on our, and their, information security systems, which attacks are of ever-increasing levels of sophistication and are made by groups and individuals with a wide range of motives and expertise, including organized criminal groups, hacktivists, nation states and others. While we have invested in information technology security measures and the protection of confidential information, there can be no assurance that our efforts will prevent service interruptions or security breaches. Any such interruptions or breach may substantially impair our ability to operate our business and would compromise our, and their, networks and the information stored could be accessed, publicly disclosed, lost, or stolen. Any such access, disclosure, or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations, and damage our reputation, any of which could adversely affect our business.

**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 in February 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 has been, and is likely to remain, volatile. 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 clinical trials for EDIT-101 and any preclinical studies and clinical trials of any other 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;

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- 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, including of a Chief Financial Officer and Chief Medical Officer, 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;
- 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 or fail to regularly publish reports on us, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

***A 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 significant 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.

We have registered all shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates. In addition, under the terms of certain of our license agreements and certain promissory notes that we may issue in the future in connection with these license agreements, we may elect to issue shares of our common stock in satisfaction of specified payment obligations of ours, which shares may be subject to rights requiring us to register such shares under the Securities Act of 1933, as amended (the “Securities Act”). Such an election by us could result in the issuance of a substantial number of shares and upon registration under the Securities Act these shares would be able to be freely sold in the public market, subject to volume limitations applicable to affiliates. If any of the additional shares described above 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.

In addition, certain of our employees, executive officers, directors, and affiliated stockholders have entered or may enter into Rule 10b5-1 plans providing for sales of shares of our common stock from time to time. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the participant establishing the plan when entering into the plan, without further direction from such participant. A Rule 10b5-1 plan may be amended or terminated in some circumstances. Our employees, executive officers, directors, and affiliated stockholders also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

***We incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives and corporate governance practices.***

As a public company we have incurred, and will continue to incur, significant legal, accounting, and other expenses that we did not incur as a private company. 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 have had 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 devote a substantial amount of time towards maintaining compliance with these requirements. These requirements increase our legal and financial compliance costs and make some activities more time-consuming and costly. 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 are required to furnish a report by our management on our internal control over financial reporting and are required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To maintain compliance with SOX Section 404, we will continue to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to dedicate internal resources, 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 neither we nor our independent registered public accounting firm will 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 consolidated 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 restated certificate of incorporation and amended and restated 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 restated certificate of incorporation and amended and restated 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 amended and restated bylaws or restated certificate of incorporation;
- the ability of our board of directors to make, alter, or repeal our amended and restated 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 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 restated 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 restated 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 restated certificate of incorporation or amended and restated 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 5. Other Information**

On August 6, 2019, our board of directors (the “Board”) appointed Cynthia Collins as the President and Chief Executive Officer of our company, effective as of August 19, 2019. Ms. Collins has served on the Board since December 2018, and as our interim Chief Executive Officer since March 2019 and our President since June 2019.

In connection with her appointment, we entered into an employment offer letter with Ms. Collins, dated as of August 6, 2019 (the “Offer Letter”). The Offer Letter provides for an annual base salary of \$625,000 and an annual target bonus equal to 60% of her base salary. In addition, we have agreed to grant Ms. Collins (i) an option to purchase 500,000 shares of our common stock, which shall vest as to 25% of the shares underlying the option on the first anniversary of the date of grant and an additional 2.0833% of the shares underlying the option at the end of each successive month following such date (the “Time-Vesting Option”) until the fourth anniversary of the employment commencement date, (ii) an option to purchase 250,000 shares of our common stock, which shall vest as to one-third of the shares underlying the option on the date on which the closing price of our common stock has for 15 consecutive trading days (in the five-year period following grant) equaled or exceeded \$50.00, \$75.00 and \$100.00, respectively (the “Performance-Vesting Option”) and (iii) a restricted stock unit award for 20,000 shares of our common stock which shall vest as to one-third of the shares on each of the first, second and third anniversaries of the date of grant (the “RSU Award”). The Time-Vesting Option and the Performance-Vesting Option each will have an exercise price equal to the closing price of our common stock on the Nasdaq Global Select Market on the date of grant. Ms. Collins is entitled to severance benefits in accordance with our severance benefits plan (the “Severance Plan”), provided that a “Non-Change in Control Termination” (as defined in the Severance Plan) shall mean a termination of her employment by us without cause or by her with good reason prior to or more than twelve (12) months following a change in control (as such terms are defined in the Severance Plan) and if Ms. Collins terminates her employment for good reason or if we terminate her employment without cause at any time in a termination covered by the Severance Plan, the Time-Vesting Option and the RSU Award shall become fully vested and exercisable, or free from forfeiture or repurchase. We have also agreed to reimburse Ms. Collins for certain relocation and housing expenses and to provide a tax gross-up with respect to such expenses.

Pursuant to the terms of the consulting agreement governing her service as interim Chief Executive Officer, Ms. Collins was eligible to receive a special bonus payable in the form of an equity award upon the achievement of certain goals and at the discretion of our Organization, Leadership and Compensation Committee. On August 6, 2019, our Board approved the payment of such bonus by the grant of a restricted stock unit award for 8,283 shares of our common stock. The consulting agreement will be terminated effective upon Ms. Collins’ first day of employment with us.

The description of the Offer Letter is qualified in its entirety by the full text of the Offer Letter, a copy of which will be filed with our Quarterly Report on Form 10-Q for the quarter ended September 30, 2019.

Ms. Collins, age 61, previously served as the Chief Executive Officer of Human Longevity, Inc. (“Human

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Longevity”), a genomics company, from January 2017 to December 2017. Prior to joining Human Longevity, Ms. Collins served as the Chief Executive Officer and GM of the Cell Therapy and Lab Business division of GE Healthcare Life Sciences, a division of General Electric Company (“GE Healthcare”), a global digital industrial company, from April 2015 to December 2016, as the CEO of Clariant Diagnostics, Inc., a division of GE Healthcare, from October 2013 to April 2015, as Chief Executive Officer and director of GenVec, Inc., a public biopharmaceutical company, from May 2012 to September 2013 and as Group Vice President, Cellular Analysis of Beckman Coulter, a global supplier of diagnostic solutions, from 2007 to 2011.

**Item 6. Exhibits**

Exhibit Index

<b>Exhibit Number</b>	<b>Description of Exhibit</b>
31.1	<a href="#">Rule 13a-14(a) Certification of Principal Executive Officer</a>
31.2	<a href="#">Rule 13a-14(a) Certification of Principal Financial Officer</a>
32.1	<a href="#">Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. §1350</a>
101	The following financial statements from the Company’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2019, formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (unaudited), (ii) Consolidated Statements of Operations (unaudited), (iii) Consolidated Statements of Comprehensive Income (Loss) (unaudited), (iv) Consolidated Statement of Stockholders’ Equity (unaudited), (v) Consolidated Statements of Cash Flows (unaudited) and (vi) Notes to Condensed Consolidated Financial Statements (unaudited), tagged as blocks of text and including detailed tags.
104	The cover page from the Company’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2019, formatted in Inline XBRL.



**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: August 7, 2019

By: /s/ Cynthia Collins  
Cynthia Collins  
Chief Executive Officer  
(Principal Executive Officer)

## CERTIFICATIONS

I, Cynthia Collins, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2019

By: /s/ Cynthia Collins  
Cynthia Collins  
Principal Executive Officer

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## CERTIFICATIONS

I, Eric Ek, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2019

By: /s/ Eric Ek

Eric Ek

*Principal Financial Officer*

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**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 June 30, 2019, 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: August 7, 2019

By: /s/ Cynthia Collins  
Cynthia Collins  
Principal Executive Officer

Date: August 7, 2019

By: /s/ Eric Ek  
Eric Ek  
Principal Financial Officer

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