

**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 March 31, 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: 001-36065

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**ACCELERON PHARMA INC.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**2836**  
(Primary Standard Industrial  
Classification Code Number)

**27-0072226**  
(I.R.S. Employer  
Identification Number)

**128 Sidney Street  
Cambridge, MA 02139  
(617) 649-9200**

(Address, including zip code, and telephone number, including  
area code, of registrant's principal executive offices)

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit 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**

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.001 per share	XLRN	The Nasdaq Global Market

As of April 30, 2019, there were 52,685,740 shares of the registrant's Common Stock, par value \$0.001 per share, outstanding.

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**PART I. FINANCIAL INFORMATION****Item 1. Financial Statements**

**Acceleron Pharma Inc.**  
**Condensed Consolidated Balance Sheets**  
(amounts in thousands except share and per share data)  
(unaudited)

	March 31, 2019	December 31, 2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 214,490	\$ 144,052
Collaboration receivables (all amounts are with a related party)	2,780	7,039
Prepaid expenses and other current assets	7,897	7,662
Short-term investments	223,106	147,260
<b>Total current assets</b>	<b>448,273</b>	<b>306,013</b>
Property and equipment, net	7,027	7,106
Right-of-use - Operating leases	27,817	—
Restricted cash	1,597	1,597
Other assets	107	105
Long-term investments	75,509	—
<b>Total assets</b>	<b>\$ 560,330</b>	<b>\$ 314,821</b>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 3,930	\$ 419
Accrued expenses	13,803	18,209
Operating lease obligations, current portion	5,537	—
Deferred rent	—	284
<b>Total current liabilities</b>	<b>23,270</b>	<b>18,912</b>
Operating lease obligations, net of current portion	24,918	—
Deferred rent, net of current portion	—	2,381
Warrants to purchase common stock	1,607	1,491
<b>Total liabilities</b>	<b>49,795</b>	<b>22,784</b>
Commitments and contingencies (Note 14)		
Stockholders' equity:		
Undesignated preferred stock, \$0.001 par value: 25,000,000 shares authorized and no shares issued or outstanding	—	—
Common stock, \$0.001 par value: 175,000,000 shares authorized; 52,542,118 and 46,260,747 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	53	47
Additional paid-in capital	1,135,376	879,099
Accumulated deficit	(624,602)	(586,549)
Accumulated other comprehensive loss	(292)	(560)
<b>Total stockholders' equity</b>	<b>510,535</b>	<b>292,037</b>
<b>Total liabilities and stockholders' equity</b>	<b>\$ 560,330</b>	<b>\$ 314,821</b>

See accompanying notes to these condensed consolidated financial statements.

**Accelaron Pharma Inc.**  
**Condensed Consolidated Statements of Operations and Comprehensive Loss**  
**(amounts in thousands except per share data)**  
**(unaudited)**

	<b>Three Months Ended March 31,</b>	
	<b>2019</b>	<b>2018</b>
Revenue:		
Collaboration revenue:		
Cost-sharing, net	2,780	3,232
Total revenue (all amounts are with a related party)	2,780	3,232
Costs and expenses:		
Research and development	32,771	23,431
General and administrative	10,814	7,441
Total costs and expenses	43,585	30,872
Loss from operations	(40,805)	(27,640)
Other income, net	2,772	1,431
Loss before income taxes	(38,033)	(26,209)
Income tax provision	(20)	(10)
Net loss	\$ (38,053)	\$ (26,219)
Net loss per share- basic and diluted	\$ (0.74)	\$ (0.58)
Weighted-average number of common shares used in computing net loss per share- basic and diluted	51,126	45,516
Other comprehensive loss:		
Net loss	\$ (38,053)	\$ (26,219)
Net unrealized holding gains (losses) on short-term and long-term investments during the period, net of tax of \$76 and zero for the three months ended March 31, 2019 and 2018, respectively	268	(429)
Comprehensive loss	\$ (37,785)	\$ (26,648)

See accompanying notes to these condensed consolidated financial statements.

**Accelaron Pharma Inc.**  
**Condensed Consolidated Statements of Stockholders' Equity**  
**(amounts in thousands except share and per share data)**  
**(unaudited)**

	<u>Common Stock</u>					<b>Total Stockholders' Equity</b>
	<b>Number of Shares</b>	<b>\$0.001 Par Value</b>	<b>Additional Paid-In Capital</b>	<b>Accumulated Deficit</b>	<b>Comprehensive Loss</b>	
Balance at December 31, 2018	46,260,747	\$ 47	\$ 879,099	\$ (586,549)	\$ (560)	\$ 292,037
Stock-based compensation	—	—	6,992	—	—	6,992
Issuance of common stock, net of expense \$500	6,151,163	6	248,124	—	—	248,130
Exercise of stock options	35,919	—	766	—	—	766
Vesting of restricted stock units	75,028	—	(393)	—	—	(393)
Issuance of common stock related to ESPP	19,661	—	788	—	—	788
Unrealized gain on available-for-sale securities, net of tax	—	—	—	—	268	268
Net loss	—	—	—	(38,053)	—	(38,053)
Balance at March 31, 2019	<u>52,542,518</u>	<u>\$ 53</u>	<u>\$ 1,135,376</u>	<u>\$ (624,602)</u>	<u>\$ (292)</u>	<u>\$ 510,535</u>

	<u>Common Stock</u>					<b>Total Stockholders' Equity</b>
	<b>Number of Shares</b>	<b>\$0.001 Par Value</b>	<b>Additional Paid-In Capital</b>	<b>Accumulated Deficit</b>	<b>Comprehensive Loss</b>	
Balance at December 31, 2017	45,261,175	\$ 46	\$ 839,090	\$ (473,024)	\$ (895)	\$ 365,216
Stock-based compensation	—	—	5,696	—	—	5,696
Exercise of stock options	358,685	—	4,715	—	—	4,716
Vesting of restricted stock units	65,183	—	(363)	—	—	(363)
Issuance of common stock related to ESPP	19,556	—	662	—	—	662
Net exercise of warrants to purchase common stock	18,449	—	797	—	—	797
Unrealized loss on available-for-sale securities	—	—	—	—	(429)	(429)
Effect of adoption of ASC 606	—	—	—	3,704	—	3,704
Net loss	—	—	—	(26,219)	—	(26,219)
Balance at March 31, 2018	<u>45,723,048</u>	<u>\$ 46</u>	<u>\$ 850,597</u>	<u>\$ (495,539)</u>	<u>\$ (1,324)</u>	<u>\$ 353,780</u>

**Accelaron Pharma Inc.**  
**Condensed Consolidated Statements of Cash Flows**  
**(amounts in thousands)**  
**(unaudited)**

	Three Months Ended March 31,	
	2019	2018
<b>Operating Activities</b>		
Net loss	\$ (38,053)	\$ (26,219)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	2,168	904
Stock-based compensation	6,992	5,696
Other non-cash items	959	88
Changes in assets and liabilities:		
Prepaid expenses and other assets	(928)	(749)
Collaboration receivables (all amounts are with a related party)	4,259	338
Accounts payable	3,466	591
Accrued expenses	(4,544)	(4,233)
Operating lease obligations (Note 13)	(627)	—
Other changes in operating assets and liabilities	—	153
Net cash used in operating activities	(26,308)	(23,431)
<b>Investing Activities</b>		
Purchases of investments	(216,116)	(35,947)
Proceeds from sales and maturities of investments	64,133	24,530
Purchases of property and equipment	(587)	(504)
Net cash used in investing activities	(152,570)	(11,921)
<b>Financing Activities</b>		
Proceeds from issuance of common stock from public offering, net of issuance costs	248,155	—
Payments for capital lease expenditures	—	(78)
Net proceeds from exercises and vesting of stock awards, ESPP contributions, and exercise of warrants to purchase common stock	1,161	5,016
Net cash provided by financing activities	249,316	4,938
Net increase (decrease) in cash, cash equivalents and restricted cash	70,438	(30,414)
Cash, cash equivalents and restricted cash at beginning of period	145,649	101,282
Cash, cash equivalents and restricted cash at end of period	\$ 216,087	\$ 70,868
<b>Supplemental Disclosure of Non-Cash Investing and Financing Activities:</b>		
Purchase of property and equipment included in accounts payable and accrued expenses	\$ 267	\$ 314
Reclassification of warrant liability to additional paid-in capital	\$ —	\$ 797
Acquisition of capital lease	\$ —	\$ 139

See accompanying notes to these condensed consolidated financial statements.

**Accelaron Pharma Inc.**  
**Notes to Condensed Consolidated Financial Statements**  
**(unaudited)**

**1. Nature of Business**

Accelaron Pharma Inc. (Accelaron or the Company) is a Cambridge, Massachusetts-based clinical stage biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases. The Company's leadership in the understanding of TGF-beta biology and protein engineering generates innovative compounds that engage the body's ability to regulate cellular growth and repair.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, the risk that the Company never achieves profitability, the need for substantial additional financing, risk of relying on third parties, risks of clinical trial failures, dependence on key personnel, protection of proprietary technology and compliance with government regulations.

**2. Basis of Presentation**

The accompanying interim condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

The accompanying interim condensed consolidated financial statements are unaudited and reflect the application of certain significant accounting policies as described below and elsewhere in these notes to the financial statements. As of March 31, 2019, the Company's significant accounting policies and estimates, which are detailed in the Company's Annual Report on Form 10-K for the year ended December 31, 2018, have not changed, and the unaudited interim financial statements have been prepared on the same basis as the audited annual financial statements as of and for the year ended December 31, 2018, except for the adoption of Accounting Standards Updates (ASU) No. 2016-02, *Leases (Topic 842)*, as discussed further in Note 13. In the opinion of management, the accompanying interim condensed consolidated financial statements reflect all adjustments, consisting of normal recurring adjustments, necessary for the fair presentation of the Company's financial position as of March 31, 2019, the results of its operations for the three months ended March 31, 2019 and 2018, and its cash flows for the three months ended March 31, 2019 and 2018.

The results for the three months ended March 31, 2019 are not necessarily indicative of the results to be expected for the year ending December 31, 2019, any other interim periods, or any future year or period. These interim financial statements should be read in conjunction with the audited financial statements as of and for the year ended December 31, 2018, and the notes thereto, together with Management's Discussion and Analysis of Financial Condition and Results of Operations, contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

**3. Use of Estimates**

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts expensed during the reporting period.

Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the consolidated financial statements if these results differ from historical experience, or other assumptions do not turn out to be substantially accurate, even if such assumptions are reasonable when made. In preparing these consolidated financial statements, management used significant estimates in the following areas, among others: revenue recognition and accrued clinical expenses.

**4. Segment Information**

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker, or decision making group, in making decisions on how to allocate resources and assess performance. The Company's chief operating decision maker is the chief executive officer. The Company and the chief executive officer view the Company's operations and manage its business as one operating segment, which is the discovery, development and commercialization of highly innovative therapeutics to treat serious and rare diseases.

## **5. Cash Equivalents and Short-term and Long-term Investments**

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in interest-bearing money market accounts. Cash equivalents are carried at cost, which approximates their fair market value.

The Company determines the appropriate classification of marketable securities at the time of purchase and reevaluates such designation at each balance sheet date. The Company has classified all of its marketable securities at March 31, 2019 as "available-for-sale" pursuant to ASC 320, *Investments – Debt and Equity Securities*. The Company records available-for-sale securities at fair value, with the unrealized gains and losses included in accumulated other comprehensive income (loss) in stockholders' equity. There were no realized gains or losses on marketable securities for the three months ended March 31, 2019 and 2018.

Investments not classified as cash equivalents are presented as either short-term or long-term investments based on both their maturities as well as the time period the Company intends to hold such securities.

The Company adjusts the cost of available-for-sale debt securities for amortization of premiums and accretion of discounts to maturity. The Company includes such amortization and accretion in interest income. The cost of securities sold is based on the specific identification method. The Company includes in interest income interest and dividends on securities classified as available-for-sale.

The Company reviews marketable securities for other-than-temporary impairment whenever the fair value of a marketable security is less than the amortized cost and evidence indicates that a marketable security's carrying amount is not recoverable within a reasonable period of time. Other-than-temporary impairments of investments are recognized in the consolidated statements of operations if the Company has experienced a credit loss, has the intent to sell the marketable security, or if it is more likely than not that the Company will be required to sell the marketable security before recovery of the amortized cost basis. Evidence considered in this assessment includes reasons for the impairment, compliance with the Company's investment policy, the severity and the duration of the impairment and changes in value subsequent to the end of the period.

In March 2017, the FASB issued Accounting Standards Update 2017-08, *Receivables - Nonrefundable Fees and Other Costs (Subtopic 310-20): Premium Amortization on Purchased Callable Debt Securities* (ASU 2017-08). This standard amends the amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period to the earliest call date. The Company adopted ASU 2017-08 effective January 1, 2019 with no material impact on its consolidated financial statements and related disclosures.

The aggregate fair value of securities held by the Company in an unrealized loss position for less than twelve months as of March 31, 2019 and December 31, 2018 was \$138.2 million and \$51.2 million, respectively. The aggregate fair value of securities held by the Company in an unrealized loss position for more than twelve months as of March 31, 2019 and December 31, 2018 was \$58.2 million and \$94.3 million, respectively. The aggregate unrealized loss for those securities in an unrealized loss position for more than twelve months is \$0.1 million and \$0.4 million, respectively. The Company determined it did not hold any investments with any other-than-temporary impairment as of March 31, 2019 and December 31, 2018.

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The following is a summary of cash, cash equivalents and available-for-sale securities as of March 31, 2019 and December 31, 2018, (in thousands):

	March 31, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash and cash equivalents due in 90 days or less	\$ 214,497	\$ —	\$ (7)	\$ 214,490
Available-for-sale securities:				
Corporate obligations due in one year or less	109,578	3	(117)	109,464
Corporate obligations due in more than one year	53,539	54	(6)	53,587
U.S. Treasury securities due in one year or less	85,044	13	(31)	85,026
U.S. Treasury securities due in more than one year	13,440	14	—	13,454
Certificates of deposit due in one year or less	1,715	—	(2)	1,713
Certificates of deposit due in more than one year	490	1	—	491
Mortgage and other asset backed securities due in one year or less	26,953	—	(50)	26,903
Mortgage and other asset backed securities due in more than one year	7,969	8	—	7,977
Total available-for-sale securities	<u>\$ 298,728</u>	<u>\$ 93</u>	<u>\$ (206)</u>	<u>\$ 298,615</u>
Total cash, cash equivalents and available-for-sale securities	<u>\$ 513,225</u>	<u>\$ 93</u>	<u>\$ (213)</u>	<u>\$ 513,105</u>

  

	December 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash and cash equivalents due in 90 days or less	\$ 144,064	\$ —	\$ (12)	\$ 144,052
Available-for-sale securities:				
Corporate obligations due in one year or less	73,671	—	(267)	73,404
U.S. Treasury securities due in one year or less	45,346	—	(79)	45,267
Certificates of deposit due in one year or less	1,715	—	—	1,715
Mortgage and other asset backed securities due in one year or less	26,982	—	(108)	26,874
Total available-for-sale securities	<u>\$ 147,714</u>	<u>\$ —</u>	<u>\$ (454)</u>	<u>\$ 147,260</u>
Total cash, cash equivalents and available-for-sale securities	<u>\$ 291,778</u>	<u>\$ —</u>	<u>\$ (466)</u>	<u>\$ 291,312</u>

**6. Restricted Cash**

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the condensed consolidated balance sheet that sum to the total of the same such amounts shown in the statement of cash flows (in thousands):

	March 31,	
	2019	2018
Cash and cash equivalents	\$ 214,490	\$ 69,736
Restricted cash	1,597	1,132
Total cash, cash equivalents and restricted cash shown in the statement of cash flows	<u>\$ 216,087</u>	<u>\$ 70,868</u>

As of March 31, 2019 and December 31, 2018, the Company maintained letters of credit totaling \$1.6 million held in the form of certificates of deposit and money market funds as collateral for the Company's facility lease obligation and its credit cards.

**7. Concentrations of Credit Risk and Off-Balance Sheet Risk**

The Company has no off-balance sheet risk, such as foreign exchange contracts, option contracts, or other foreign hedging arrangements. Financial instruments that potentially subject the Company to concentrations of credit risk are primarily cash, cash equivalents, restricted cash, short-term and long-term investments and receivables. The Company maintains its cash and cash equivalent balances and short-term and long-term investments with financial institutions that management believes are

creditworthy. Short-term and long-term investments consist of investment grade corporate obligations, treasury notes, asset backed securities, and certificates of deposit. The Company's investment policy includes guidelines on the quality of the institutions and financial instruments and defines allowable investments that the Company believes minimizes the exposure to concentrations of credit risk.

The Company routinely assesses the creditworthiness of its customers and collaboration partners. The Company has not experienced any material losses related to receivables from individual customers and collaboration partners, or groups of customers. The Company does not require collateral. Due to these factors, no additional credit risk beyond amounts provided for collection losses is believed by management to be probable in the Company's collaboration receivables.

## 8. Fair Value Measurements

The following tables set forth the Company's financial instruments carried at fair value using the lowest level of input applicable to each financial instrument as of March 31, 2019 and December 31, 2018 (in thousands):

	March 31, 2019			Total
	Quoted Prices in Active Markets for Identical Items (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Assets:				
Money market funds	\$ 136,520	\$ —	\$ —	\$ 136,520
Corporate obligations	—	193,017	—	193,017
U.S. Treasury securities	—	118,976	—	118,976
Certificates of deposit	—	2,204	—	2,204
Mortgage and other asset backed securities	—	34,881	—	34,881
Total assets	\$ 136,520	\$ 349,078	\$ —	\$ 485,598
Liabilities:				
Warrants to purchase common stock	\$ —	\$ —	\$ 1,607	\$ 1,607
Total liabilities	\$ —	\$ —	\$ 1,607	\$ 1,607

	December 31, 2018			Total
	Quoted Prices in Active Markets for Identical Items (Level 1)	Significant other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Assets:				
Money market funds	\$ 74,023	\$ —	\$ —	\$ 74,023
Corporate obligations	—	128,920	—	128,920
U.S. Treasury securities	—	56,978	—	56,978
Certificates of deposit	—	1,715	—	1,715
Mortgage and other asset backed securities	—	26,874	—	26,874
Total assets	\$ 74,023	\$ 214,487	\$ —	\$ 288,510
Liabilities:				
Warrants to purchase common stock	\$ —	\$ —	\$ 1,491	\$ 1,491
Total liabilities	\$ —	\$ —	\$ 1,491	\$ 1,491

The money market funds noted above are included in cash and cash equivalents in the accompanying condensed consolidated balance sheets. The Company recognizes transfers between levels of the fair value hierarchy as of the end of the reporting period. There were no transfers within the hierarchy during the three months ended March 31, 2019 or the year ended December 31, 2018.

Items measured at fair value on a recurring basis include short-term and long-term investments (Note 5), and warrants to purchase common stock (Note 12). During the periods presented, the Company has not changed the manner in which it values assets and liabilities that are measured at fair value using Level 3 inputs.

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The following table sets forth a summary of changes in the fair value of the Company's common stock warrant liabilities, which represent a recurring measurement that is classified within Level 3 of the fair value hierarchy, wherein fair value is estimated using significant unobservable inputs (in thousands):

	Three Months Ended March 31,	
	2019	2018
Beginning balance	\$ 1,491	\$ 2,236
Change in fair value	116	(119)
Exercises	—	(797)
Ending balance	\$ 1,607	\$ 1,320

The fair value of the warrants to purchase common stock on the date of issuance and on each re-measurement date for those warrants classified as liabilities was estimated using either the Monte Carlo simulation framework, which incorporates future financing events over the remaining life of the warrants to purchase common stock, or for certain re-measurement dates, due to the warrants being deeply in the money, the Black-Scholes option pricing model. Due to the nature of these inputs, the valuation of the warrants is considered a Level 3 measurement. At each reporting period, the Company evaluates the best valuation methodology. At March 31, 2019, the Black-Scholes option pricing model was used.

The Company measures eligible assets and liabilities at fair value, with changes in value recognized in earnings. Fair value treatment may be elected either upon initial recognition of an eligible asset or liability or, for an existing asset or liability, if an event triggers a new basis of accounting. The Company did not elect to re-measure any of its existing financial assets or liabilities, and did not elect the fair value option for any financial assets and liabilities transacted in the three months ended March 31, 2019 or the year ended December 31, 2018.

## 9. Net Loss Per Share

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

	Three Months Ended March 31,	
	2019	2018
Outstanding stock options	4,115	3,718
Common stock warrants	39	39
Shares issuable under employee stock purchase plan	13	13
Outstanding restricted stock units (1)	630	631
	4,797	4,401

(1) This balance is comprised of both the restricted stock units and performance-based restricted stock units described in Note 17.

## 10. Comprehensive Loss

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions, other events, and circumstances from non-owner sources. Comprehensive loss consists of net loss and other comprehensive loss, which includes certain changes in equity that are excluded from net loss. Comprehensive loss has been disclosed in the accompanying consolidated statements of operations and comprehensive loss. Accumulated other comprehensive loss is presented separately on the consolidated balance sheets and consists entirely of unrealized holding gains and losses on investments as of March 31, 2019 and December 31, 2018.

## 11. Recent Accounting Pronouncements - Not Yet Adopted

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board (FASB) or other standard setting bodies and adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its financial position or results of operations upon adoption.

## 12. Warrants

Below is a summary of the number of shares issuable upon exercise of outstanding warrants and the terms and accounting treatment for the outstanding warrants (in thousands, except per share data):

	Warrants as of		Weighted-Average Exercise Price Per Share	Expiration	Balance Sheet Classification	
	March 31, 2019	December 31, 2018			March 31, 2019	December 31, 2018
Warrants to purchase common stock	39	39	\$ 5.88	June 10, 2020 - July 9, 2020	Liability	Liability
All warrants	39	39	\$ 5.88			

### 13. Leases

In February 2016, the FASB issued Accounting Standards Codification Topic 842, *Leases* (ASC 842), which replaces the existing guidance for leases. ASC 842 requires lessees to recognize assets and liabilities on the balance sheet for the rights and obligations created by all leases with terms of more than 12 months. ASC 842 also requires certain qualitative and quantitative disclosures designed to give financial statement users information on the amount, timing, and uncertainty of cash flows arising from leases.

The Company has adopted ASC 842 effective January 1, 2019. The Company has elected to employ the transitional relief recently offered by the FASB under ASU 2018-11 and implement the new standard without the restatement of comparative periods' financial information. ASU-2018-11 also provides for recognizing the effects of applying ASC 842 as a cumulative-effect adjustment to retained earnings as of January 1, 2019; however, no such adjustment was recorded as of January 1, 2019.

The Company has elected to employ the package of practical expedients offered under ASC 842, which allow the Company to not reassess the following:

- the presence of a lease in any expired or existing contracts;
- the lease classification for any existing or expired leases; and
- the initial direct costs for any existing leases.

The Company currently leases approximately 125,000 square feet of office and laboratory space in five adjacent buildings in Cambridge, Massachusetts (the Leases). The Leases were classified as operating leases under ASC 840. The Leases are also classified as operating leases under ASC 842 in accordance with the Company's election of the practical expedient under ASC 842. Pursuant to the package of practical expedients, the Company will also not reassess initial direct costs for the Leases. Additionally, the Company elected to account for the lease components and non-lease components as a single lease component.

The Company occupied the premises of the Leases at various points in time prior to January 1, 2019 under non-cancelable agreements which expire at various dates through September 2023. Each of the Leases have options to renew for periods ranging from three to five years, which are not included in the measurement of these leases. All of the Company's leases contain escalating rent clauses, which require higher rent payments in future years. There are no variable payments, exercise purchase options, penalties, fees, or residual value guarantees under the Leases. The Company is also obligated to pay the Landlord for certain costs, taxes, and operating expenses related to the premises. However, the Company has concluded that these payments are not in-substance fixed payments and therefore are not included in the calculation of the related lease liability and asset under ASC 842.

The Company recorded the liability associated with the Leases at the present value of the lease payments not yet paid, discounted using the discount rate for the Leases established at the adoption date. As the discount rate implicit in the Leases was typically not readily determinable, the Company utilized its incremental borrowing rate. In transition to ASC 842, the Company utilized the remaining lease term of its leases in determining the appropriate incremental borrowing rates.

The IBR for the Leases was determined by establishing a credit rating of the Company using the Ordered Logit (oLogit) model. The oLogit Model is a quantitative way to assess the credit rating of a company. Based on the established credit rating, the Company determined a borrowing rate using regression analysis on selected financial ratios of publicly traded comparable companies and the companies' credit ratings, adjusted for the risk-free rate, which resulted in an IBR of approximately 10%.

The Company recorded the liability associated with the Leases at the present value of the lease payments not yet paid, discounted using the incremental borrowing rate for the each lease established at the adoption date. On January 1, 2019, the Company recorded a right-of-use asset in the amount of \$29.1 million, which represents a lease liability of \$31.0 million, adjusted for previously recognized lease-related balances, including deferred rent of \$2.7 million and prepaid rent of \$0.7

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million. This lease liability will be reduced over the remaining lease term based on cash payments made offset by accretion of monthly interest calculated on the lease liability. The right-of-use asset will be amortized over the remaining lease term in an amount equal to the difference between the calculated straight-line expense of the total lease payments less the monthly interest calculated on the remaining lease liability.

The Company will recognize rent expense, calculated as the remaining cost of the lease allocated over the remaining lease term on a straight-line basis. Rent expense will be presented as part of continuing operations in the condensed statement of consolidated operations and comprehensive loss. For the three months ended March 31, 2019, the Company recognized rent expense of \$2.0 million.

For the three months ended March 31, 2019, the Company paid \$2.1 million in rent relating to the Leases. As payments resulting from an operating lease, the \$2.1 million is classified within operating activities in the condensed consolidated statements of cash flows.

The following table contains supplemental balance sheet information pertaining to the Company's leases for the three months ended March 31, 2019:

	<u>Three Months Ended March 31,</u> <u>2019</u>
Weighted average remaining lease term	3.8 years
Weighted average discount rate	10.58%

Future minimum lease payments under the Company's non-cancelable operating leases as of March 31, 2019 are as follows, (in thousands):

	<u>As of March 31,</u> <u>2019</u>
2019	\$ 6,292
2020	8,610
2021	8,336
2022	8,409
2023	6,450
Total Lease Payments	\$ 38,097
Less: imputed interest	(7,642)
Total	<u>\$ 30,455</u>

#### 14. Commitments and Contingencies

##### Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of its business. The Company was not subject to any material legal proceedings during the three months ended March 31, 2019, and, to the best of its knowledge, no material legal proceedings are currently pending or threatened.

##### Other

The Company is also party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at March 31, 2019 and December 31, 2018, or royalties on future sales of specified products. No milestones or royalty payments under these agreements are expected to be payable in the immediate future. See Note 15 for discussion of these arrangements.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company indemnifies, holds harmless, and agrees to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners or customers, in connection with any U.S. patent

or any copyright or other intellectual property infringement claim by any third party with respect to the Company's products. The term of these indemnification agreements is generally perpetual any time after execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

## **15. Significant Agreements**

### **Celgene**

#### ***Overview***

On February 20, 2008, the Company entered into an agreement with Celgene relating to sotatercept (the Original Sotatercept Agreement), which was amended on August 2, 2011 (as amended, the Amended Sotatercept Agreement). The Company further amended and restated the Original Sotatercept Agreement in its entirety on September 18, 2017, (the Restated Sotatercept Agreement). On August 2, 2011, the Company entered into a second agreement with Celgene for luspatercept, (the Luspatercept Agreement).

Since December 31, 2018, there have been no material changes to the key terms of the above agreements. For further information on the terms of the agreements, please see the notes to the consolidated financial statements included in the Company's Form 10-K for the year ended December 31, 2018.

#### ***Restated Sotatercept Agreement***

The Restated Sotatercept Agreement provides Celgene with an exclusive license to sotatercept outside of the field of pulmonary hypertension, referred to as the PH field, and provides the Company with the worldwide rights to develop and commercialize sotatercept in the PH field.

In connection with the Restated Sotatercept Agreement, Celgene agreed not to develop or commercialize in the PH field any compound developed under the Restated Sotatercept Agreement or the Luspatercept Agreement, and the Company agreed not to develop or commercialize any compound developed under the Restated Sotatercept Agreement or the Luspatercept Agreement in any field outside the PH field. The Company has the right to license, transfer, or sell its rights to develop and commercialize sotatercept in the PH field, subject to Celgene's first right of negotiation.

#### ***Luspatercept Agreement***

Under the terms of the Luspatercept Agreement, the Company and Celgene collaborate worldwide for the joint development and commercialization of luspatercept. The Company also granted Celgene an option for future products for which the Company files an Investigational New Drug application for the treatment of anemia.

The Company retained responsibility for research and development through the end of Phase 1 and the Company's initial luspatercept beta-thalassemia and luspatercept MDS Phase 2 clinical trials, as well as manufacturing the clinical supplies for these studies. Celgene will conduct subsequent Phase 2 and Phase 3 clinical studies and will be responsible for overseeing the manufacture of Phase 3 and commercial supplies by third party contract manufacturing organizations.

Beginning in November 2013, the Company agreed to conduct certain extension studies for the benefit of the luspatercept program, which included certain clinical and non-clinical services. These studies were mutually agreed to by both parties and are directed by the Joint Development Committee. The Company is reimbursed for these services under the same terms and rates of the existing agreements.

#### ***Both Agreements***

Under both agreements, Celgene is responsible for paying 100% of worldwide development costs and 100% of any commercialization costs worldwide for sotatercept (outside of the pulmonary hypertension field) and luspatercept. The Company has the right to co-promote sotatercept (outside of the pulmonary hypertension field), luspatercept and future products in North America, for which the commercialization costs provided in the commercialization plan and budget as approved by the Joint Commercialization Committee will be entirely funded by Celgene. Additionally, from time to time the Company may elect to conduct additional activities to support commercialization of luspatercept at its own expense. The Company will receive tiered royalties in the low-to-mid 20% range on net sales of sotatercept (outside of the pulmonary hypertension field) and luspatercept, and these royalty schedules are the same for both agreements.

### *Accounting Analysis*

As of the ASC 606 adoption date, the only remaining undelivered element was participation in the JDC. The transaction price allocated to participation in the JDC based on the established standalone selling price of all performance obligations was de minimis as the sotatercept and luspatercept licenses carried the most significant portion of the value included in the agreements, and the Company's remaining effort on the JDC is minimal. Therefore, the Company recorded a cumulative-effect reduction to opening accumulated deficit of \$3.7 million as the adoption date and a corresponding decrease to deferred revenue, of which \$0.5 million was recorded to current deferred revenue and \$3.2 million was recorded to long-term deferred revenue.

The next likely milestone payment for luspatercept would be \$25.0 million and result from U.S. Food and Drug Administration (FDA) or European Medicines Agency (EMA) acceptance of a Biologics Licensing Application or equivalent for luspatercept in either myelodysplastic syndromes or beta-thalassemia. The Company and Celgene submitted market applications for luspatercept in the United States and Europe in April 2019. Following application submission, the FDA will determine the acceptance of the application for "filing" by 60 days from the submission date. Similarly, the EMA will validate the application within 10 days of submission. In accordance with the Company's accounting policy regarding revenue recognition, the revenue associated with this milestone will be recognized once it is probable that the applications are accepted for review by either the FDA or EMA. Milestone payments that are not within the control of the Company or the licensee are not considered probable of being achieved until those approvals are received. The acceptance of the application by either the FDA or EMA could occur as early as the second quarter of 2019, which would result in the recognition of the milestone during that period. As of March 31, 2019, the acceptance of the application is not within the control of the Company or the licensee, and therefore, the Company could not determine if it is probable that a regulatory agency will accept the application.

Through March 31, 2019, under all Celgene arrangements the Company has received net cost-share payments and milestones of \$116.6 million and \$44.6 million for luspatercept and sotatercept, respectively. The Company recorded net cost-sharing revenue of \$2.8 million and \$3.2 million during the three months ended March 31, 2019 and 2018, respectively.

### **Other Agreements**

#### *Other*

In 2004, the Company entered into a license agreement with a non-profit institution for an exclusive, sublicensable, worldwide, royalty-bearing license to certain patents developed by the institution (Primary Licensed Products). In addition, the Company was granted a non-exclusive, non-sub-licensable license for Secondary Licensed Products. As compensation for the licenses, the Company issued 62,500 shares of its common stock to the institution, the fair value of which was \$25,000, and was expensed during 2004, to research and development expense. The Company also agreed to pay specified development milestone payments totaling up to \$2.0 million for sotatercept and \$0.7 million for luspatercept. In addition, the Company is obligated to pay milestone fees based on the Company's research and development progress, and U.S. sublicensing revenue ranging from 10%-25%, as well as royalties ranging from 1.0%-3.5% of net sales on any products under the licenses. During the three months ended March 31, 2019 and 2018, the Company did not reach any milestones defined under the agreement, and, therefore, no amounts have been paid or expensed.

In May 2014, the Company executed a collaboration agreement with a research technology company. The Company paid an upfront research fee of \$0.3 million upon execution of the agreement. The Company also received an option to obtain a commercial license to the molecules developed during the collaboration. During the three months ended March 31, 2019 and 2018, the Company expensed \$0.2 million and zero, respectively, of milestones and fees defined under the agreement.

### **16. Stockholders' Equity**

On January 18, 2019, the Company completed the sale of its underwritten public offering of 5,348,838 shares of common stock at public offering price of \$43.00 per share, resulting in net proceeds of \$215.8 million. In connection with the January 2019 public offering, on February 12, 2019, the underwriters fully exercised their over-allotment option to purchase an additional 802,325 shares of the Company's common stock. The total net proceeds from the January 2019 public offering and the underwriters' exercise of their option to purchase additional shares of common stock was \$248.2 million.

### **17. Stock-Based Compensation**

The Company recognized stock-based compensation expense related to the 2003 Stock Option and Restricted Stock Plan (the 2003 Plan), the 2013 Equity Incentive Plan (the 2013 Plan), and the 2013 Employee Stock Purchase Plan (the 2013 ESPP) in the consolidated statements of operations and comprehensive loss during the three months ended March 31, 2019 and 2018, respectively, as follows (in thousands):

	Three Months Ended March 31,	
	2019	2018
Research and development	\$ 3,500	\$ 2,869
General and administrative	3,492	2,827
	<u>\$ 6,992</u>	<u>\$ 5,696</u>

### Stock Options

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

	Three Months Ended March 31,	
	2019	2018
Expected volatility	59.1%	63.0%
Expected term (in years)	6.0	6.0
Risk-free interest rate	2.6%	2.6%
Expected dividend yield	—%	—%

The following table summarizes the stock option activity under the Company's stock option plans during the three months ended March 31, 2019 (in thousands, except per share amounts and years):

	Number of Stock Options	Weighted- Average Exercise Price Per Share	Weighted- Average Contractual Life (in years)	Aggregate Intrinsic Value(1)
Outstanding at December 31, 2018	3,513	\$ 34.40	7.31	
Granted	663	\$ 41.88		
Exercised	(36)	\$ 21.56		
Canceled or forfeited	(25)	\$ 41.60		
Outstanding at March 31, 2019	<u>4,115</u>	\$ 35.67	7.16	\$ 45,481
Exercisable at March 31, 2019	<u>2,202</u>	\$ 32.57	6.20	\$ 30,869

- (1) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in the money at March 31, 2019.

During the three months ended March 31, 2019, current and former employees of the Company exercised a total of 35,519 options, resulting in total proceeds of \$0.8 million.

The aggregate intrinsic value of options exercised during the three months ended March 31, 2019 was \$0.9 million.

As of March 31, 2019, there was \$39.9 million of unrecognized compensation expense related to unvested stock options that is expected to be recognized over a weighted-average period of 2.73 years.

### Restricted Stock Units

The following table summarizes the restricted stock unit (RSU) activity under the 2013 Plan during the three months ended March 31, 2019 (in thousands, except per share amounts):

	Number of Stock Units	Weighted- Average Grant Date Fair Value Per Share
Unvested balance at December 31, 2018	372	\$ 36.53
Granted	113	\$ 41.70
Vested	(84)	\$ 34.20
Forfeited	(7)	\$ 35.99
Unvested balance at March 31, 2019	<u>394</u>	<u>\$ 38.51</u>



As of March 31, 2019, there was approximately \$12.4 million of related unrecognized compensation cost, which the Company expects to recognize over a remaining weighted-average period of 1.96 years.

### Performance-Based Restricted Stock Units

The Company has granted performance-based restricted stock units (PSU) whereby vesting accelerates upon the occurrence of certain milestone events. In September 2019, any of these PSUs that remain unvested will vest. When achievement of a milestone becomes probable, compensation cost is recognized from the grant date through the estimated date of achievement. If achievement is not considered probable the expense is recognized from the grant date through September 2019. The following table summarizes PSU activity under the 2013 Plan during the three months ended March 31, 2019 (in thousands, except per share amounts):

	Number of Stock Units	Weighted- Average Grant Date Fair Value Per Share
Unvested balance at December 31, 2018	236	\$ 31.42
Granted	—	\$ —
Vested	—	\$ —
Forfeited	—	\$ —
Unvested balance at March 31, 2019	236	\$ 31.42

As of March 31, 2019, there was approximately \$0.4 million of related unrecognized compensation cost, which the Company expects to recognize over a remaining weighted-average period of 0.19 years.

### Employee Stock Purchase Plan

During the three months ended March 31, 2019 and 2018, the Company recorded \$0.1 million and \$0.1 million, respectively, of stock-based compensation expense related to the 2013 ESPP.

### 18. Income Taxes

Deferred tax assets and deferred tax liabilities are recognized based on temporary differences between the financial reporting and tax basis of assets and liabilities using statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized. Due to the uncertainty surrounding the realization of the favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company's otherwise recognizable net deferred tax assets.

### 19. Related Party Transactions

#### Celgene Corporation

In connection with the Company's January 2019 public offering, Celgene purchased 706,206 shares of common stock. In connection with this and prior transactions, Celgene owned 12.0% and 12.2% of the Company's fully diluted equity as of March 31, 2019 and December 31, 2018, respectively. Refer to Note 15 for additional information regarding this collaboration arrangement.

During the three months ended March 31, 2019 and 2018, all revenue recognized by the Company was recognized under the Celgene collaboration arrangement and, as of March 31, 2019, the Company had no deferred revenue related to the Celgene collaboration arrangement.

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

*The following information should be read in conjunction with the unaudited condensed consolidated financial statements and the notes thereto included in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2018.*

*Certain matters discussed in this Quarterly Report on Form 10-Q may be deemed to be forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. In this Quarterly Report on Form 10-Q, words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "project," "should," "strategy," "target," "vision," "will," "would," or, in each case, the negative or other variations thereon or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.*

*The forward-looking statements in this Quarterly Report on Form 10-Q include, among other things, statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things:*

- our ongoing and planned preclinical studies and clinical trials;*
- clinical trial data and the timing of results of our ongoing clinical trials;*
- our plans to develop and commercialize ACE-083 and our preclinical therapeutic candidates;*
- our and Celgene's plans to develop and commercialize luspatercept and sotatercept;*
- the potential benefits of strategic partnership agreements and our ability to enter into selective strategic partnership arrangements;*
- the timing of anticipated milestone payments under our collaboration agreements with Celgene;*
- the timing of, and our and Celgene's ability to, obtain and maintain regulatory approvals for our therapeutic candidates;*
- the rate and degree of market acceptance and clinical utility of any approved therapeutic candidate, particularly in specific patient populations;*
- our ability to quickly and efficiently identify and develop therapeutic candidates;*
- our manufacturing capabilities and strategy;*
- our plans for commercialization and marketing;*
- our intellectual property position; and*
- our estimates regarding our results of operations, financial condition, liquidity, capital requirements, prospects, growth and strategies.*

*By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics, and industry changes and depend on the economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and events in the industry in which we operate may differ materially from the forward-looking statements contained herein.*

*Any forward-looking statements that we make in this Quarterly Report on Form 10-Q speak only as of the date of such statements, and we undertake no obligation to update such statements to reflect events or circumstances after the date of this Quarterly Report on Form 10-Q or to reflect the occurrence of unanticipated events.*

*You should also read carefully the factors described in the section "Item 1A. Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2018 to better understand the risks and uncertainties inherent in our business and underlying any forward-looking statements. You are advised, however, to consult any further disclosures we make on related subjects in our subsequent Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, press releases, and our website.*

## Overview

We are a leading biopharmaceutical company in the discovery and development of TGF-beta superfamily therapeutics to treat serious and rare diseases. Our research focuses on key natural regulators of cellular growth and repair, particularly the Transforming Growth Factor-Beta, or TGF-beta, protein superfamily. By combining our discovery and development expertise, including our proprietary knowledge of the TGF-beta superfamily, and our internal protein engineering and manufacturing capabilities, we have generated several innovative therapeutic candidates, all of which encompass novel potential first-in-class mechanisms of action. We have focused and prioritized our research and development activities within three key therapeutic areas: hematologic, neuromuscular and pulmonary. If successful, these candidates could have the potential to significantly improve clinical outcomes for patients across these areas of high, unmet need.

Luspatercept, our lead program, and sotatercept, are partnered with Celgene Corporation, or Celgene. Luspatercept is an erythroid maturation agent designed to promote red blood cell production through a novel mechanism, and is being developed to treat chronic anemia and associated complications in myelodysplastic syndromes, or MDS, beta-thalassemia, and myelofibrosis. In 2018, we and Celgene announced positive results for two Phase 3 clinical trials with luspatercept; one for the treatment of patients with lower-risk MDS, known as the MEDALIST trial, and another for the treatment of patients with transfusion-dependent beta-thalassemia, also known as the BELIEVE trial. In the MEDALIST trial, luspatercept achieved a highly statistically significant improvement in the primary endpoint of red blood cell (RBC) transfusion independence of at least 8 consecutive weeks during the first 24 weeks compared to placebo. In the BELIEVE trial, luspatercept achieved a highly statistically significant improvement in the primary endpoint of erythroid response, which was defined as at least a 33 percent reduction from baseline in red blood cell (RBC) transfusion burden with a reduction of at least 2 units during the protocol-defined period of 12 consecutive weeks, from week 13 to week 24, compared to placebo. Results from the MEDALIST and BELIEVE trials were then presented during plenary and oral sessions, respectively, at the 60th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2018, and we expect to submit these results for publication during 2019. Both of these presentations were selected for "Best of ASH," chosen from among the thousands of meeting abstracts as "the biggest breakthroughs from the meeting's scientific presentations." In April 2019, Celgene submitted regulatory applications for luspatercept in both MDS and beta-thalassemia in the United States and Europe based on the safety and efficacy results of the MEDALIST and BELIEVE trials.

Celgene is also currently conducting a Phase 2 clinical trial with luspatercept in non-transfusion-dependent beta-thalassemia patients, referred to as the "BEYOND" trial, with preliminary top-line results currently expected in 2020. Celgene has also initiated a Phase 3 clinical trial, the "COMMANDS" trial, in first-line, lower-risk MDS patients and enrollment is ongoing. Enrollment is also currently ongoing in a Phase 2 clinical trial being conducted by Celgene for the treatment of patients with myelofibrosis, a rare bone marrow disorder, with preliminary top-line results currently expected in the second half of 2019. If approved in the United States and Europe, we believe that there is an annual peak sales opportunity for luspatercept in excess of \$2 billion in lower-risk MDS and beta-thalassemia, and upon successful development and approval in the United States and Europe, an additional \$1 billion in myelofibrosis. We and Celgene are evaluating luspatercept for the treatment of anemia in potential new indications that could provide additional sales opportunities.

For sotatercept, we have the rights to fund, develop, and lead the global commercialization of sotatercept in pulmonary hypertension, which we refer to as the PH field, including pulmonary arterial hypertension, or PAH. PAH is a rare and chronic, rapidly progressing disorder characterized by the constriction of small pulmonary arteries, resulting in abnormally high blood pressure in the pulmonary arteries. We are currently enrolling the PULSAR Phase 2 clinical trial of sotatercept for the treatment of patients with PAH with preliminary results expected in the first half of 2020. We are also currently enrolling an exploratory study called SPECTRA to provide us with greater understanding of sotatercept's potential impact on PAH, with preliminary results expected in 2020. If sotatercept is commercialized to treat PAH and we recognize such revenue, then Celgene will be eligible to receive a royalty in the low 20% range on global net sales. In certain circumstances Celgene may recognize revenue related to the commercialization of sotatercept in PAH, and in this scenario we will be eligible to receive a royalty from Celgene such that the economic position of the parties is equivalent to the scenario in which we recognize such revenue.

For luspatercept and, outside of the PH field, sotatercept, Celgene is responsible for paying 100% of the development costs for all clinical trials. We may receive a maximum of \$545.0 million for the potential development, regulatory and commercial milestone payments. If luspatercept and, outside of the PH field, sotatercept, are commercialized, we are eligible to receive a royalty on net sales in the low-to-mid 20% range and we have a co-promotion right in North America, for which our commercialization costs provided in the commercialization plan and budget as approved by the Joint Commercialization Committee will be entirely funded by Celgene. From time to time we may elect to conduct additional activities to support commercialization of luspatercept at our own expense.

We are independently developing our wholly-owned neuromuscular candidate, ACE-083. ACE-083 is designed for the treatment of focal muscle disorders, and we are currently conducting Phase 2 clinical trials with ACE-083 in patients with facioscapulohumeral dystrophy, or FSHD, as well as in patients with Charcot-Marie-Tooth disease, or CMT. We previously

announced results from part 1 of each of our Phase 2 clinical trials in patients with FSHD and CMT showing increases in mean total and contractile muscle volume, reductions in fat fraction, and an encouraging safety profile. Enrollment is complete in part 2 of the ACE-083 Phase 2 clinical trial in patients with FSHD and is ongoing in the ACE-083 Phase 2 clinical trial in patients with CMT. We expect to announce preliminary results from part 2 of each of these Phase 2 clinical trials by the second half of 2019 for FSHD and by the first quarter of 2020 for CMT.

In addition to our clinical programs, we are conducting research within our three focused disease areas - hematologic, neuromuscular and pulmonary - in order to identify new therapeutic candidates to advance into clinical trials.

As of March 31, 2019, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$773.8 million from public investors, \$154.1 million in equity investments from our collaboration partners and \$291.3 million in upfront payments, milestones, and net research and development payments from our collaboration partners.

We expect to continue to incur significant expenses and increasing operating losses over at least the next several years. We expect our expenses will increase substantially in connection with our ongoing activities, if and as we:

- conduct clinical trials for ACE-083, sotatercept in the PH field, or any future therapeutic candidates;
- continue our preclinical studies and potential clinical development efforts of our existing preclinical therapeutic candidates;
- continue research activities for the discovery of new therapeutic candidates;
- manufacture therapeutic candidates for our preclinical studies and clinical trials;
- acquire or in-license other therapeutic candidates and patents; and
- seek regulatory approval for our therapeutic candidates.

We will not generate revenue from product sales unless and until we or a partner successfully complete development and obtain regulatory approval for one or more of our therapeutic candidates, and this is subject to significant uncertainty. All current and future development and commercialization costs for luspatercept and, outside of the PH field, sotatercept, as agreed to between us and Celgene are paid by Celgene. From time to time we may elect to conduct additional activities to support luspatercept at our own expense. If we obtain regulatory approval for ACE-083, sotatercept in the PH field, or any future therapeutic candidate, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such costs are not paid by future partners. We will seek to fund our operations through the sale of equity, debt financings or other sources, including potential additional collaborations. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such other arrangements as, and when, needed, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our therapeutic candidates.

Our ability to generate product revenue and become profitable depends upon our and our partners' ability to successfully commercialize products. We expect to incur losses for the foreseeable future, and we expect these losses to increase as we continue our development of, and seek regulatory approvals for, our therapeutic candidates and potentially begin to commercialize any approved products. For a description of the numerous risks and uncertainties associated with product development, see "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2018.

## **Financial Operations Overview**

### **Revenue**

#### ***Collaboration Revenue***

We have not generated any revenue from the sale of products. Our revenue to date has been predominantly derived from collaboration revenue, which includes license and milestone revenues and cost-sharing revenue, generated through collaboration and license agreements with partners for the development and commercialization of our therapeutic candidates. Cost-sharing revenue represents amounts reimbursed by our collaboration partners for expenses incurred by us for research and development activities and, potentially, co-promotion activities, under our collaboration agreements. Cost-sharing revenue is recognized in the period that the related activities are performed.

### **Costs and Expenses**

#### ***Research and Development Expenses***

Research and development expenses consist primarily of costs directly incurred by us for the development of our therapeutic candidates, which include:

- direct employee-related expenses, including salaries, benefits, travel and stock-based compensation expense of our research and development personnel;
- expenses incurred under agreements with clinical research organizations, or CROs, and investigative sites that will conduct our clinical trials;
- the cost of acquiring and manufacturing preclinical and clinical study materials and developing manufacturing processes;
- allocated facilities, depreciation, and other expenses, which include rent and maintenance of facilities, insurance and other supplies;
- expenses associated with obtaining and maintaining patents; and
- costs associated with preclinical activities and regulatory compliance.

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites.

We cannot determine with certainty the duration and completion costs of the current or future clinical trials of our therapeutic candidates or if, when, or to what extent we will generate revenues from the commercialization and sale of any of our therapeutic candidates for which we or any partner obtain regulatory approval. We or our partners may never succeed in achieving regulatory approval for any of our therapeutic candidates. The duration, costs and timing of clinical trials and development of our therapeutic candidates will depend on a variety of factors, including:

- the scope, rate of progress, and expense of our ongoing, as well as any additional, clinical trials and other research and development activities;
- future clinical trial results;
- potential changes in government regulation; and
- the timing and receipt of any regulatory approvals.

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

From inception through March 31, 2019, we have incurred \$691.5 million in research and development expenses. We plan to increase our research and development expenses for the foreseeable future as we continue the development of our TGF-beta platform therapeutic candidates, the discovery and development of preclinical therapeutic candidates, and the development of our clinical programs. Expenses associated with luspatercept and, outside of the PH field, sotatercept, are generally reimbursed 100% by Celgene. These reimbursements are recorded as revenue. We are expensing the costs of a Phase 1 clinical trial for ACE-2494, and Phase 2 clinical trials for luspatercept, sotatercept, and ACE-083, of which the luspatercept trials are reimbursed by Celgene. Our Phase 1 clinical trial for ACE-2494 is being discontinued. With respect to the luspatercept Phase 3 clinical trials directly conducted by Celgene, we do not incur and are not reimbursed for expenses related to these development activities.

We manage certain activities such as clinical trial operations, manufacture of therapeutic candidates, and preclinical animal toxicology studies through third-party CROs. The only costs we track by each therapeutic candidate are external costs such as services provided to us by CROs, manufacturing of preclinical and clinical drug product, and other outsourced research and development expenses. We do not assign or allocate to individual development programs internal costs such as salaries and benefits, facilities costs, lab supplies and the costs of preclinical research and studies, except for luspatercept costs for the purposes of billing Celgene. Our external research and development expenses during the three months ended March 31, 2019 and 2018 are as follows:

(in thousands)	Three Months Ended March 31,	
	2019	2018
Luspatercept(1)	1,339	1,613
Sotatercept(2)	4,091	1,559
ACE-083	4,250	2,583
ACE-2494(3)	769	598
Total direct research and development expenses	10,449	6,353
Other expenses(4)	22,322	17,078
Total research and development expenses	\$ 32,771	\$ 23,431

- (1) These expenses associated with luspatercept are reimbursed 100% by Celgene.
- (2) These expenses are associated with our development of sotatercept in pulmonary arterial hypertension.
- (3) Development of ACE-2494 is being discontinued. We expect to incur all remaining material expense by the end of 2019.
- (4) Other expenses include employee and unallocated contractor-related expenses, facility expenses, lab supplies, and miscellaneous expenses.

#### ***General and Administrative Expenses***

General and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation and travel expenses for our employees in executive, operational, finance and human resource functions and other general and administrative expenses including directors' fees and professional fees for accounting and legal services.

We continue to incur expenses related to audit, legal, regulatory and tax-related services associated with maintaining compliance with exchange listing and Securities and Exchange Commission, or SEC, requirements, director and officer insurance premiums, and investor relations costs associated with being a public company. We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our therapeutic candidates. Additionally, if and when we believe regulatory approval of a therapeutic candidate appears likely, to the extent that we are undertaking commercialization of such therapeutic candidate ourselves, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations.

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

Other income (expense), net consists primarily of the interest income earned on cash, cash equivalents and investments.

#### **Critical Accounting Policies and Estimates**

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

During the three months ended March 31, 2019, there have been no material changes to our critical accounting policies as reported in our Annual Report on the Form 10-K for the year ended December 31, 2018. For further information on our critical and other significant accounting policies, including the adoption of ASC 842, see the notes to the condensed consolidated financial statements 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.

## Results of Operations

### Comparison of the Three Months Ended March 31, 2019 and 2018

(in thousands)	Three Months Ended March 31,		Increase (Decrease)
	2019	2018	
Revenue:			
Collaboration revenue:			
Cost-sharing, net	2,780	3,232	(452)
Total revenue	2,780	3,232	(452)
Costs and expenses:			
Research and development	32,771	23,431	9,340
General and administrative	10,814	7,441	3,373
Total costs and expenses	43,585	30,872	12,713
Loss from operations	(40,805)	(27,640)	(13,165)
Other income, net	2,772	1,431	1,341
Loss before income taxes	(38,033)	(26,209)	(11,824)
Income tax benefit	(20)	(10)	(10)
Net loss	\$ (38,053)	\$ (26,219)	\$ (11,834)

**Revenue.** We recognized revenue of \$2.8 million in the three months ended March 31, 2019, compared to \$3.2 million in the same period in 2018. All of the revenue in both periods was derived from the Celgene agreements. This \$0.4 million decrease is primarily due to the decreased clinical activities for the luspatercept Phase 2 clinical trials.

**Research and Development Expenses.** Research and development expenses were \$32.8 million in the three months ended March 31, 2019, compared to \$23.4 million in the same period in 2018. This \$9.4 million increase is primarily related to growth in order to support our wholly-owned therapeutic candidates and preclinical programs and includes:

- an increase in personnel expenses of \$2.5 million;
- an increase in external clinical trial expenses of \$3.9 million related to our sotatercept and ACE-083 Phase 2 clinical trials;
- an increase in toxicology expenses of \$1.8 million related to the advancement of our clinical and preclinical programs; and
- an increase in drug supply and miscellaneous research expense of \$1.2 million.

**General and Administrative Expenses.** General and administrative expenses were \$10.8 million in the three months ended March 31, 2019, compared to \$7.4 million in the same period in 2018. The \$3.4 million increase is primarily due to the following factors:

- an increase in personnel expense of \$2.9 million related to increased headcount to support our growth; and
- an increase in professional fees of \$0.5 million.

**Other Income, Net.** Other income, net was \$2.8 million in the three months ended March 31, 2019, compared to \$1.4 million for the same period in 2018. This \$1.4 million change was primarily due to a \$1.3 million increase in the interest earned on our investment portfolio as a result of our higher balance of interest-bearing cash equivalents and short- and long-term investments.

**Income Tax Provision.** Income tax provision is attributable to the realization of current year losses that offset unrealized gains, recognized in other comprehensive income, from our investment portfolio.

### Liquidity and Capital Resources

We have incurred losses and cumulative negative cash flows from operations since our inception in June 2003, and as of March 31, 2019, we had an accumulated deficit of \$624.6 million. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of the sale of equity, debt financings or other sources, including potential additional collaborations.

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As of March 31, 2019, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$773.8 million from public investors, \$154.1 million in equity investments from our collaboration partners and \$291.3 million in upfront payments, milestones, and net research and development payments from our collaboration partners.

As of March 31, 2019, we had \$513.1 million in cash, cash equivalents and investments. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

## Cash Flows

The following table sets forth the primary sources and uses of cash for each of the periods set forth below (in thousands):

(in thousands)	Three Months Ended March 31,	
	2019	2018
Net cash (used in) provided by:		
Operating activities	\$ (26,308)	\$ (23,431)
Investing activities	(152,570)	(11,921)
Financing activities	249,316	4,938
Net increase (decrease) in cash, cash equivalents and restricted cash	<u>\$ 70,438</u>	<u>\$ (30,414)</u>

### Operating Activities

Net cash used in operating activities was \$26.3 million for the three months ended March 31, 2019 compared to \$23.4 million during the same period in 2018. Net cash used in operating activities has increased over this period as a result of increased headcount and related facilities, as well as additional external expenses to support our wholly-owned therapeutic programs. The change primarily consists of the following:

- for the three months ended March 31, 2019, a net loss of \$38.1 million adjusted for non-cash items of \$10.1 million and a net increase in operating assets and liabilities of \$1.7 million. Non-cash items consist primarily of stock-based compensation expense of \$7.0 million, depreciation and amortization expense of \$2.2 million, and other non-cash items of \$0.9 million. The net increase in operating assets and liabilities consists primarily of an increase in accounts payable of \$3.5 million and a decrease in collaboration receivables of \$4.3 million, offset by a decrease in accrued expenses of \$4.5 million and a decrease in other net operating assets and liabilities of \$1.6 million.
- for the three months ended March 31, 2018, a net loss of \$26.2 million adjusted for non-cash items of \$6.9 million and a net increase in operating assets and liabilities of \$3.9 million. Non-cash items consist primarily of stock-based compensation expense of \$5.7 million and depreciation and amortization expense of \$0.9 million. The net decrease in operating assets and liabilities consists primarily of a decrease in accrued expenses of \$4.2 million.

### Investing Activities

Net cash used in investing activities was \$152.6 million for the three months ended March 31, 2019 compared to \$11.9 million during the same period in 2018. Net cash used in investing activities primarily consisted of the following amounts relating to activity within our investment portfolio:

- for the three months ended March 31, 2019, net purchases of investments of \$152.0 million; and
- for the three months ended March 31, 2018, net purchases of investments of \$11.4 million.

Net purchases during the periods were due to the execution of our investment strategy in accordance with our policy as we began to invest the money raised in our September 2017 and January 2019 public offerings, respectively, in marketable securities.

### Financing Activities

Net cash provided by financing activities was \$249.3 million for the three months ended March 31, 2019 compared to \$4.9 million for the same period in 2018. Net cash provided by financing activities primarily consisted primarily of the following:

- for the three months ended March 31, 2019, net proceeds of \$248.2 million from our January 2019 public offering and the underwriters full exercise of the over-allotment option in the offering, as well as \$1.2 million in cash proceeds from the exercise of stock options and the issuance of common stock related to the employee stock purchase plan; and

- for the three months ended March 31, 2018, \$5.4 million in cash proceeds from the exercise of stock options and the issuance of common stock related to the employee stock purchase plan.

### **Operating Capital Requirements**

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We will not generate revenue from product sales unless and until we or our partners obtain regulatory approval of and commercialize one of our current or future therapeutic candidates. We anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek and obtain regulatory approvals for ACE-083, sotatercept in the PH field and any future therapeutic candidates, and begin to commercialize any approved products. We are subject to all of the risks inherent in the development of therapeutic candidates, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. We have incurred, and expect to continue to incur, additional costs associated with operating as a public company. We anticipate that we will need additional funding in connection with our continuing operations.

Based on our current operating plan and projections, we believe that our current cash, cash equivalents and investments will be sufficient to fund our projected operating requirements until such time as we expect to receive significant royalty revenue from luspatercept sales. However, if there are changes in our operating plan or projections, we may need to raise additional funds for future development and operational plans and activities.

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

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

- the achievement of milestones and royalties under our agreement with Celgene;
- the terms and timing of any other collaborative, licensing and other arrangements that we may establish;
- the initiation, progress, timing and completion of preclinical studies and clinical trials for our therapeutic candidates and potential therapeutic candidates;
- the number and characteristics of therapeutic candidates that we pursue;
- the progress, costs and results of our clinical trials;
- the outcome, timing and cost of regulatory approvals;
- delays that may be caused by changing regulatory requirements;
- the cost and timing of hiring new employees to support our continued growth;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;
- the costs and timing of procuring clinical and commercial supplies of our therapeutic candidates;

- the extent to which we acquire or invest in businesses, products or technologies; and
- the costs involved in defending and prosecuting litigation regarding in-licensed intellectual property.

#### **Net Operating Loss (NOL) Carryforwards**

We had deferred tax assets of approximately \$183.0 million as of December 31, 2018, which have been fully offset by a valuation allowance due to uncertainties surrounding our ability to realize these tax benefits. The deferred tax assets are primarily composed of federal and state tax net operating loss, or NOL, carryforwards, research and development tax credit carryforwards, and deferred revenue, accruals, and other temporary differences. As of December 31, 2018, we had federal NOL carryforwards of approximately \$556.0 million and state NOL carryforwards of \$515.0 million available to reduce future taxable income, if any. Of these federal and state NOL carryforwards, \$438.0 million and \$515 million, respectively, will expire at various times through 2038. The federal NOL of \$118.0 million generated in 2018 can be carried forward indefinitely. In general, if we experience a greater than 50 percent aggregate change in ownership of certain significant stockholders over a three-year period, or a Section 382 ownership change, utilization of our pre-change NOL carryforwards are subject to an annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended, and similar state laws. Such limitations may result in expiration of a portion of the NOL carryforwards before utilization and may be substantial. If we experience a Section 382 ownership change in connection with our public offerings or as a result of future changes in our stock ownership, some of which changes are outside our control, the tax benefits related to the NOL carryforwards may be limited or lost. For additional information about our taxes, see Note 13 to the financial statements in our Annual Report on Form 10-K for the year ended December 31, 2018.

#### **Contractual Obligations and Commitments**

During the three months ended March 31, 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” included in our Annual Report on Form 10-K for the year ended December 31, 2018.

#### **Recent Accounting Pronouncements**

For information on recent accounting pronouncements, see Recently Issued and Adopted Accounting Standards in the notes to the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

#### **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 in the rules and regulations of the Securities and Exchange Commission.

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

We are exposed to market risk related to changes in interest rates. As of March 31, 2019 and December 31, 2018, we had cash, cash equivalents and investments of \$513.1 million and \$291.3 million, respectively. Our cash equivalents are invested primarily in bank deposits and money market mutual funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Our investments are subject to interest rate risk and could fall in value if market interest rates increase. Due to the duration of our investment portfolio and the low risk profile of our investments, we do not believe an immediate 100 basis point change in interest rates would have a material effect on the fair market value of our portfolio. We have the ability to hold our investments until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

We contract with CROs and manufacturers internationally. Transactions with these providers are predominantly settled in U.S. dollars and, therefore, we believe that we have only minimal exposure to foreign currency exchange risks. We do not hedge against foreign currency risks.

### **Item 4. Controls and Procedures**

#### **Management’s Evaluation of our Disclosure Controls and Procedures**

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities Exchange Act of 1934, or the Exchange Act, is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC’s rules and forms, and (2) accumulated and

communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of March 31, 2019, management, with the participation of our Chief Executive Officer and Chief Financial Officer, performed an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act. Our disclosure controls and procedures are designed to ensure that information required to be disclosed in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including the Chief Executive Officer and the Chief Financial Officer, to allow timely decisions regarding required disclosures. Any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objective. Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of March 31, 2019, the design and operation of our disclosure controls and procedures were effective.

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

During the quarter ended March 31, 2019, we implemented certain internal controls in connection with our adoption of Topic ASC 842 and the implementation of a new ERP system. There were no other changes in our internal control over financial reporting (as defined in Rule 13a-15(f) under the Exchange Act) during the fiscal quarter covered by this report 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

While we are not currently a party to any material legal proceedings, we could become subject to legal proceedings in the ordinary course of business. We do not expect any such potential items to have a significant impact on our financial position.

### Item 1A. Risk Factors

There have been no material changes from the risk factors previously disclosed in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2018.

### Item 6. Exhibits

<u>Exhibit Number</u>	<u>Description of Exhibit</u>
31.1	<a href="#">Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</a>
31.2	<a href="#">Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</a>
32.1	<a href="#">Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</a>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document

**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.

**ACCELERON PHARMA INC.**

Date: May 9, 2019

By: /s/ HABIB J. DABLE  
Habib J. Dable  
*Chief Executive Officer and President*

Date: May 9, 2019

By: /s/ KEVIN F. MCLAUGHLIN  
Kevin F. McLaughlin  
*Senior Vice President, Chief Financial Officer and Treasurer*

## CERTIFICATION OF CHIEF EXECUTIVE OFFICER, ACCELERON PHARMA INC.

I, Habib J. Dable, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Acceleron Pharma 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(s) 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(s) 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 function):
  - 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.

May 9, 2019

/s/ Habib J. Dable

Date

Habib J. Dable  
Chief Executive Officer and President  
(Principal Executive Officer)

## CERTIFICATION OF CHIEF FINANCIAL OFFICER, ACCELERON PHARMA INC.

I, Kevin F. McLaughlin, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Acceleron Pharma 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(s) 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(s) 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 function):
  - 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.

May 9, 2019

Date

/s/ Kevin F. McLaughlin

Kevin F. McLaughlin  
Senior Vice President, Chief Financial Officer and Treasurer  
(Principal Financial Officer)

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

In connection with the Quarterly Report on Form 10-Q of Acceleron Pharma Inc. (the "Company") for the period ended March 31, 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, that to his or her knowledge:

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

Date: May 9, 2019

By: /s/ Habib J. Dable  
Habib J. Dable  
President and Chief Executive Officer  
(Principal Executive Officer)

Date: May 9, 2019

By: /s/ Kevin F. McLaughlin  
Kevin F. McLaughlin  
Senior Vice President, Chief Financial Officer and Treasurer  
(Principal Financial Officer)