

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

---

**FORM 10-Q**

---

(Mark One)

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

For the quarterly period ended **March 31, 2018**

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**

---

**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)

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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"/> (Do not check if a smaller reporting company)	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**

As of April 30, 2018, there were 45,775,325 shares of the registrant's Common Stock, par value \$0.001 per share, outstanding.

---

**TABLE OF CONTENTS**

	<u>Page</u>
<b><u>PART I. FINANCIAL INFORMATION</u></b>	<b><u>3</u></b>
<u>Item 1.</u> <u>Financial Statements (unaudited)</u>	<u>3</u>
<u>Condensed Consolidated Balance Sheets as of March 31, 2018 and December 31, 2017</u>	<u>3</u>
<u>Condensed Consolidated Statements of Operations and Comprehensive Loss for the three months ended March 31, 2018 and 2017</u>	<u>4</u>
<u>Condensed Consolidated Statements of Cash Flows for the three months ended March 31, 2018 and 2017</u>	<u>5</u>
<u>Notes to Condensed Consolidated Financial Statements</u>	<u>6</u>
<u>Item 2.</u> <u>Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>	<u>21</u>
<u>Item 3.</u> <u>Quantitative and Qualitative Disclosures About Market Risks</u>	<u>29</u>
<u>Item 4.</u> <u>Controls and Procedures</u>	<u>29</u>
<b><u>PART II. OTHER INFORMATION</u></b>	<b><u>31</u></b>
<u>Item 1.</u> <u>Legal Proceedings</u>	<u>31</u>
<u>Item 1A.</u> <u>Risk Factors</u>	<u>31</u>
<u>Item 6.</u> <u>Exhibits</u>	<u>31</u>
<b><u>SIGNATURES</u></b>	<b><u>32</u></b>

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

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

	March 31, 2018	December 31, 2017
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 69,736	\$ 100,150
Collaboration receivables (all amounts are with related party)	3,232	3,570
Prepaid expenses and other current assets	5,203	4,446
Short-term investments	222,119	177,077
Total current assets	300,290	285,243
Property and equipment, net	7,019	6,966
Restricted cash	1,132	1,132
Other assets	105	113
Long-term investments	61,488	95,723
Total assets	\$ 370,034	\$ 389,177
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 1,677	\$ 1,086
Accrued expenses	11,079	14,936
Deferred revenue	—	541
Deferred rent	186	182
Total current liabilities	12,942	16,745
Deferred revenue, net of current portion	—	3,161
Deferred rent, net of current portion	1,992	1,818
Warrants to purchase common stock	1,320	2,236
Total liabilities	16,254	23,960
Commitments and contingencies (Note 13)		
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; 45,723,048 and 45,261,175 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively	46	46
Additional paid-in capital	850,597	839,090
Accumulated deficit	(495,539)	(473,024)
Accumulated other comprehensive loss	(1,324)	(895)
Total stockholders' equity	353,780	365,217
Total liabilities and stockholders' equity	\$ 370,034	\$ 389,177

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>2018</b>	<b>2017</b>
Revenue:		
Collaboration revenue:		
License and milestone	\$ —	\$ 135
Cost-sharing, net	3,232	3,570
Total revenue (all amounts are with related party)	3,232	3,705
Costs and expenses:		
Research and development	23,431	21,727
General and administrative	7,441	7,836
Total costs and expenses	30,872	29,563
Loss from operations	(27,640)	(25,858)
Other income (expense), net	119	(45)
Interest income	1,312	502
Total other income, net	1,431	457
Loss before income taxes	(26,209)	(25,401)
Income tax provision	(10)	(6)
Net loss	\$ (26,219)	\$ (25,407)
Net loss per share- basic and diluted	\$ (0.58)	\$ (0.66)
Weighted-average number of common shares used in computing net loss per share- basic and diluted	45,516	38,404
Other comprehensive loss:		
Net loss	\$ (26,219)	\$ (25,407)
Net unrealized holding (losses) gains on short-term and long-term investments during the period	(429)	25
Comprehensive loss	\$ (26,648)	\$ (25,382)

See accompanying notes to these condensed consolidated financial statements.

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

	<b>Three Months Ended March 31,</b>	
	<b>2018</b>	<b>2017</b>
<b>Operating Activities</b>		
Net loss	\$ (26,219)	\$ (25,407)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	904	541
Stock-based compensation	5,696	6,583
Change in fair value of warrants	(119)	45
Other non-cash items	207	88
Changes in assets and liabilities:		
Prepaid expenses and other assets	(749)	(222)
Collaboration receivables (all amounts are with a related party)	338	(336)
Accounts payable	591	(778)
Accrued expenses	(4,233)	(1,951)
Deferred revenue	—	(137)
Deferred rent	153	(189)
Net cash used in operating activities	<u>(23,431)</u>	<u>(21,763)</u>
<b>Investing Activities</b>		
Purchases of investments	(35,947)	(221)
Proceeds from sales and maturities of investments	24,530	33,533
Purchases of property and equipment	(504)	(677)
Net cash (used in) provided by investing activities	<u>(11,921)</u>	<u>32,635</u>
<b>Financing Activities</b>		
Payments for withholding taxes on restricted stock units	(363)	(140)
Payments for capital lease expenditures	(78)	—
Proceeds from exercise of stock options and warrants to purchase common stock	4,716	1,151
Proceeds from issuances of common stock related to employee stock purchase plan	663	510
Net cash provided by financing activities	<u>4,938</u>	<u>1,521</u>
Net (decrease) increase in cash, cash equivalents and restricted cash	<u>(30,414)</u>	<u>12,393</u>
Cash, cash equivalents and restricted cash at beginning of period	101,282	21,896
Cash, cash equivalents and restricted cash at end of period	<u>\$ 70,868</u>	<u>\$ 34,289</u>
<b>Supplemental Disclosure of Non-Cash Investing and Financing Activities:</b>		
Purchase of property and equipment included in accounts payable and accrued expenses	<u>\$ 314</u>	<u>\$ 1,769</u>
Reclassification of warrant liability to additional paid-in capital	<u>\$ 797</u>	<u>\$ —</u>
Acquisition of capital lease	<u>\$ 139</u>	<u>\$ —</u>

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. 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, 2017 except for the adoption of Accounting Standards Update (ASU) No. 2016-18, *Restricted Cash*, which did not have a material impact, and Topic No. 606, *Revenue from Contracts with Customers*, as discussed below, and, in the opinion of management, reflect all adjustments, consisting of normal recurring adjustments, necessary for the fair presentation of the Company's financial position as of March 31, 2018, the results of its operations for the three months ended March 31, 2018 and 2017, and its cash flows for the three months ended March 31, 2018 and 2017.

The results for the three months ended March 31, 2018 are not necessarily indicative of the results to be expected for the year ending December 31, 2018, 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, 2017, 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, 2017.

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. Subsequent events have been evaluated as required. The Company has evaluated all subsequent events and determined that there are no material recognized or unrecognized events requiring disclosure, other than those disclosed in this Report on Form 10-Q.

The accompanying interim condensed consolidated financial statements reflect the application of certain significant accounting policies as described below and elsewhere in these notes to the financial statements. As of March 31, 2018, 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, 2017, have not changed, except for the adoption of ASU No. 2016-18, *Restricted Cash*, which is discussed further in Note 6, and Accounting Standards Codification Topic No. 606, *Revenue from Contracts with Customers*, which is discussed further below.

**Revenue Recognition**

Effective January 1, 2018, the Company adopted Accounting Standards Codification Topic 606, *Revenue from Contracts with Customers*, (ASC 606), using the modified retrospective transition method. Under this method, results for reporting periods beginning January 1, 2018 are presented under ASC 606, while prior period amounts are not adjusted and continue to be reported in accordance with ASC 605.

The Company has primarily generated revenue through collaboration, license and research arrangements, which are within the scope of ASC 606, with collaboration partners for the development and commercialization of therapeutic candidates. The arrangements generally contain performance obligations, which may include (1) licenses, or options to obtain licenses, to

## [Table of Contents](#)

the Company's technology, (2) research and development activities performed for the collaboration partners (3) participation on joint development committees (JDCs), and (4) the manufacturing of clinical or preclinical material. Payments pursuant to these arrangements typically include non-refundable, upfront payments, milestone payments upon achieving significant development events, research and development reimbursements, sales milestones, exercises of options, and royalties on future product sales.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in the Company's consolidated balance sheets. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, current portion. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion. Amounts recognized as revenue, but not yet received or invoiced are generally recognized as contract assets, including collaboration receivables.

To determine revenue recognition for arrangements within the scope of ASC 606, the Company performs the following five steps: (i) identify the contract(s) with the customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract, determines those that are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. Depending on the nature of the performance obligation these assessments require management to make significant judgments and estimates.

### *Exclusive Licenses*

If the license to the Company's intellectual property is determined to be distinct from the other promises or performance obligations identified in the arrangement, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred and the customer is able to use and benefit from the license. In order to assess whether the license is distinct, the Company considers the capabilities of the collaboration partner and the availability of the necessary expertise in the general marketplace to determine whether the collaboration partner can benefit from the license for its intended purpose without the receipt of the remaining elements. For licenses determined not to be distinct the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, are subject to estimates by management and may change over the course of the research and development and licensing agreement.

### *Research and Development Services*

The promises under the Company's collaboration and license agreements generally include research and development services to be performed by the Company on behalf of the collaboration partner. As the provision of research and development services is a part of the Company's central operations, when the Company is principally responsible for the performance of these services under the agreements, the Company recognizes revenue on a gross basis for research and development services in accordance with the ASC 606 framework described above.

### *Customer Options*

The Company's agreements may contain options which provide the collaboration partner the right to obtain additional licenses. If an arrangement is determined to contain customer options, the goods and services underlying the customer options are not considered to be performance obligations at the inception of the arrangement, and the associated option fees are not included in the transaction price. The Company evaluates the customer options to determine if they represent material rights, which may include options to acquire additional goods or services for free or at a discount. If the customer options are determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the arrangement. The Company allocates the transaction price to material rights based on the relative standalone selling price, which is determined based on the identified discount and the probability that the customer will exercise the option. Amounts allocated to a material right are not recognized as revenue until, at the earliest, the option is exercised.

### *Milestone Payments*

At the inception of each arrangement that includes milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks

that must be overcome to achieve the respective milestone in making this assessment. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment. If a milestone or other variable consideration relates specifically to the Company's efforts to satisfy a single performance obligation or to a specific outcome from satisfying the performance obligation, the Company generally allocates the milestone amount entirely to that performance obligation.

#### *Royalties*

For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its licensing arrangements.

For a complete discussion of accounting for collaboration revenues, see Note 14.

### **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, stock-based compensation expense, the determination of the fair value of stock-based awards, the fair value of liability-classified warrants, and accrued 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, 2018 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, 2018 and 2017.

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.

The aggregate fair value of securities held by the Company in an unrealized loss position for less than twelve months as of March 31, 2018 and December 31, 2017 was \$215.3 million and \$193.6 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, 2018 and December 31, 2017 was \$60.4 million and \$67.0 million, respectively. The aggregate unrealized loss for those securities in an unrealized loss position for more than twelve months is \$0.3 million and \$0.3 million, respectively. As a result, the Company determined it did not hold any investments with any other-than-temporary impairment as of March 31, 2018 and December 31, 2017.

[Table of Contents](#)

The following is a summary of cash, cash equivalents and available-for-sale securities as of March 31, 2018 and December 31, 2017 (in thousands):

	March 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash and cash equivalents due in 90 days or less	\$ 69,740	\$ —	\$ (4)	\$ 69,736
Available-for-sale securities:				
Corporate obligations due in one year or less	120,811	—	(456)	120,355
Corporate obligations due in more than one year	33,406	—	(347)	33,059
U.S. Treasury securities due in one year or less	50,399	—	(133)	50,266
U.S. Treasury securities due in more than one year	9,949	—	(55)	9,894
Certificates of deposit due in one year or less	7,140	—	—	7,140
Certificates of deposit due in more than one year	1,715	—	—	1,715
Mortgage and other asset backed securities due in one year or less	44,548	—	(191)	44,357
Mortgage and other asset backed securities due in more than one year	16,959	—	(138)	16,821
Total available-for-sale securities	\$ 284,927	\$ —	\$ (1,320)	\$ 283,607
Total cash, cash equivalents and available-for-sale securities	\$ 354,667	\$ —	\$ (1,324)	\$ 353,343

	December 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash and cash equivalents due in 90 days or less	\$ 100,150	\$ —	\$ —	\$ 100,150
Available-for-sale securities:				
Corporate obligations due in one year or less	99,792	—	(219)	99,573
Corporate obligations due in more than one year	57,537	—	(261)	57,276
U.S. Treasury securities due in one year or less	27,987	—	(93)	27,894
U.S. Treasury securities due in more than one year	9,968	—	(48)	9,920
Certificates of deposit due in one year or less	10,529	—	—	10,529
Certificates of deposit due in more than one year	1,715	—	—	1,715
Mortgage and other asset backed securities due in one year or less	39,236	—	(155)	39,081
Mortgage and other asset backed securities due in more than one year	26,931	—	(119)	26,812
Total available-for-sale securities	\$ 273,695	\$ —	\$ (895)	\$ 272,800
Total cash, cash equivalents and available-for-sale securities	\$ 373,845	\$ —	\$ (895)	\$ 372,950

## 6. Restricted Cash

On January 1, 2018, the Company adopted ASU 2016-18, *Statement of Cash Flows - Restricted Cash (Topic 230)*. This new standard requires companies to include amounts generally described as restricted cash and restricted cash equivalents in cash and cash equivalents when reconciling beginning-of-period and end-of-period total amounts shown on the statement of cash flows. As a result of the adoption, there was no impact to cash flows from investing or financing activities for the three months ended March 31, 2018 and March 31, 2017. \$1.1 million of restricted cash related to collateral for the Company's facility lease obligation and its credit cards, which was previously reported as an adjustment to net loss in cash flows used in operating activities for the three months ended March 31, 2017, is no longer presented within the net change in cash, cash equivalents, and restricted cash, as it is considered part of cash, cash equivalents, and 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,	
	2018	2017
Cash and cash equivalents	\$ 69,736	\$ 33,157
Restricted cash	1,132	1,132
Total cash, cash equivalents and restricted cash show in the statement of cash flows	\$ 70,868	\$ 34,289

As of March 31, 2018 and December 31, 2017, the Company maintained letters of credit totaling \$1.1 million and \$1.1 million, respectively, 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 collaboration 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, 2018 and December 31, 2017 (in thousands):

	March 31, 2018			Total
	Quoted Prices in Active Markets for Identical Items (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
<b>Assets:</b>				
Money market funds	\$ 51,974	\$ —	\$ —	\$ 51,974
Corporate obligations	—	164,549	—	164,549
U.S. Treasury securities	—	60,159	—	60,159
Certificates of deposit	—	8,855	—	8,855
Mortgage and other asset backed securities	—	62,677	—	62,677
Restricted cash	1,132	—	—	1,132
<b>Total assets</b>	<b>\$ 53,106</b>	<b>\$ 296,240</b>	<b>\$ —</b>	<b>\$ 349,346</b>
<b>Liabilities:</b>				
Warrants to purchase common stock	\$ —	\$ —	\$ 1,320	\$ 1,320
<b>Total liabilities</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ 1,320</b>	<b>\$ 1,320</b>

	December 31, 2017			Total
	Quoted Prices in Active Markets for Identical Items (Level 1)	Significant other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
<b>Assets:</b>				
Money market funds	\$ 90,702	\$ —	\$ —	\$ 90,702
Corporate obligations	—	158,849	—	158,849
U.S. Treasury securities	—	37,813	—	37,813
Certificates of deposit	—	12,244	—	12,244
Mortgage and other asset backed securities	—	67,888	—	67,888
Restricted cash	1,132	—	—	1,132
<b>Total assets</b>	<b>\$ 91,834</b>	<b>\$ 276,794</b>	<b>\$ —</b>	<b>\$ 368,628</b>
<b>Liabilities:</b>				
Warrants to purchase common stock	\$ —	\$ —	\$ 2,236	\$ 2,236
<b>Total liabilities</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ 2,236</b>	<b>\$ 2,236</b>

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, 2018 or the year ended December 31, 2017.

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.

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,	
	2018	2017
Beginning balance	\$ 2,236	\$ 1,244
Change in fair value	(119)	45
Exercises	(797)	—
Ending balance	<u>\$ 1,320</u>	<u>\$ 1,289</u>

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, 2018, 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, 2018 or the year ended December 31, 2017.

## 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,	
	2018	2017
Outstanding stock options	3,718	3,583
Common stock warrants	39	63
Shares issuable under employee stock purchase plan	13	15
Outstanding restricted stock units	631	807
	<u>4,401</u>	<u>4,468</u>

## 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, 2018 and December 31, 2017.

## 11. Recent Accounting Pronouncements

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.

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842), Amendments to the FASB Accounting Standards Codification*, which replaces the existing guidance for leases. ASU 2016-02 requires the identification of arrangements that should be accounted for as leases by lessees. In general, for lease arrangements exceeding a twelve month term, these arrangements must now be recognized as assets and liabilities on the balance sheet of the lessee. Under ASU 2016-02, a right-of-use asset and lease obligation will be recorded for all leases, whether operating or financing, while the income statement will reflect lease expense for operating leases and amortization/interest expense for financing leases. The balance sheet amount recorded for existing leases at the date of adoption of ASU 2016-02 must be calculated using the applicable incremental borrowing rate at the date of adoption. In addition, ASU 2016-02 requires the use of the modified retrospective method, which will require adjustment to all comparative periods presented in the consolidated financial statements. This guidance is effective for annual and interim periods beginning after December 15, 2018 and requires retrospective application. The Company is

currently assessing the impact that adopting ASU 2016-02 will have on its consolidated financial statements and related disclosures.

In March 2017, the FASB issued ASU 2017-08, *Receivables - Nonrefundable Fees and Other Costs (Subtopic 310-20): Premium Amortization on Purchased Callable Debt Securities*. This new standard shortens the amortization period for certain callable debt securities held at a premium. Specifically, the amendment requires the premium to be amortized to the earliest call date. The amendment does not require an accounting change for securities held at a discount; the discount continues to be amortized to maturity. The guidance is effective for annual and interim periods beginning after December 15, 2018, and early adoption is permitted. The amendment should be applied on a modified retrospective basis, with the cumulative-effect adjustment directly to retained earnings as of the beginning of the period of adoption. The Company is currently assessing the impact that adopting ASU 2017-08 will have on its consolidated financial statements and related disclosures.

## 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, 2018	December 31, 2017			March 31, 2018	December 31, 2017
Warrants to purchase common stock	39	61	\$ 5.88	June 10, 2020 - July 9, 2020	Liability (1)	Liability (1)
All warrants	39	61	\$ 5.88			

- (1) In January 2018, warrant holders exercised warrants to purchase 21,258 shares of Common Stock on a net basis, resulting in the issuance of 18,449 shares of Common Stock.

In connection with the Series E redeemable convertible preferred stock (Series E Preferred Stock) financing transactions that took place in June 2010 and July 2010, the Company issued warrants to purchase up to 871,580 shares of common stock. Each warrant was immediately exercisable and expires ten years from the original date of issuance. The warrants to purchase shares of the Company's common stock have an exercise price equal to the estimated fair value of the underlying instrument as of the initial date such warrants were issued. Each warrant is exercisable on either a physical settlement or net share settlement basis from the date of issuance. The warrant agreement contains a provision requiring an adjustment to the number of shares in the event the Company issues common stock, or securities convertible into or exercisable for common stock, at a price per share lower than the warrant exercise price. The Company concluded the anti-dilution feature required the warrants to be classified as liabilities under ASC Topic 815, *Derivatives and Hedging—Contracts in Entity's Own Equity* (ASC 815). The warrants are measured at fair value, with changes in fair value recognized as a gain or loss to other income (expense) in the statements of operations and comprehensive loss for each reporting period thereafter. The fair value of the common stock warrants was recorded as a discount to the preferred stock issued, and the preferred stock was accreted to the redemption value. At the end of each reporting period, the Company re-measured the fair value of the outstanding warrants, using current assumptions, resulting in a decrease in fair value of \$0.1 million and an increase in fair value of \$45 thousand for the three months ended March 31, 2018 and 2017, respectively, which was recorded in other income (expense) in the accompanying consolidated statements of operations and comprehensive loss. The Company will continue to re-measure the fair value of the liability associated with the warrants to purchase common stock at the end of each reporting period until the earlier of the exercise or the expiration of the applicable warrants. All remaining outstanding warrants were fully vested and exercisable as of March 31, 2018 and December 31, 2017.

## 13. 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, 2018, 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 been met at March 31, 2018 and December 31, 2017, 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 14 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.

#### **14. 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 on 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, 2017, 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, 2017.

###### *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.

The Company retained responsibility for research and development of sotatercept through the end of Phase 2a clinical trials, as well as manufacturing the clinical supplies for these trials. These activities were substantially completed in 2011. Outside of pulmonary hypertension, Celgene will also be responsible for any Phase 3 clinical trials, as well as additional Phase 2 clinical trials, and is responsible for manufacturing or overseeing the manufacture of Phase 3 and commercial supplies.

###### *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 JDC. 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. 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**

On January 1, 2018, the Company adopted ASC 606, *Revenue from Contracts with Customers*, using the modified retrospective transition method, and has elected to use the practical expedient related to contract modifications that is permitted under the rules of adoption. The practical expedient included in the transition guidance allows companies to determine and allocate the transaction price of a modified contract as of the beginning of the earliest period presented instead of requiring them to separately evaluate the effects of every modification of the contract and eliminates the requirement to retrospectively restate a contract that has been modified at the date of adoption as is generally required under ASC 606. This practical expedient was applied in the assessment of the Restated Sotatercept Agreement and Luspatercept Agreement as of the date of adoption.

The Company identified the following material promises under the Restated Sotatercept Agreement and Luspatercept Agreement: (1) licenses to develop and commercialize sotatercept and luspatercept; (2) performance of research and development services; (3) participation in the JDCs; and (4) the performance of the manufacturing services. The Company determined that the licenses to sotatercept and luspatercept technology, the research and development activities, participation in the JDCs and the manufacturing services are each distinct performance obligations. The option rights to future products related to the treatment of anemia under the Luspatercept Agreement are not considered to represent a material right as this right is a protective provision akin to exclusivity and does not represent a customer option to receive the rights or services at a discount. In addition, the Company is under no obligation to discover, develop, or deliver any new compounds that modulate anemia. Therefore, the option right under the Luspatercept Agreement is not a performance obligation. Commercialization support for each of sotatercept and luspatercept is considered to be a participatory right and not a performance obligation. The Company concluded that services provided for the extension studies do not represent a contract modification or a performance obligation but rather a separate services arrangement, which is accounted for as a separate contract. Each study includes one promise, the completion of the study, which is distinct from the performance obligations in the Restated Sotatercept Agreement and Luspatercept Agreement that is satisfied over time, and the consideration for each study approximates the stand-alone selling price. Revenue is recognized as the services for each study are provided.

Future potential milestone payments were excluded from the transaction price as they are still subject to completion of on-going clinical studies or other risks that are outside of the Company's control and therefore the risk of significant reversal has not been resolved. The next likely clinical milestone payment for luspatercept would be \$25.0 million and result from U.S. Food and Drug Administration or European Medical Association acceptance of a Biologics Licensing Application or equivalent for luspatercept in either myelodysplastic syndromes or beta-thalassemia. The next likely clinical milestone payment for sotatercept would be \$10.0 million and result from Celgene's start of a Phase 3 study with sotatercept outside of the PH field.

The transaction price includes the following payments received under the Restated Sotatercept and Luspatercept Agreement through the adoption date of December 31, 2017 for a total of \$192.3 million, as follows:

- \$25.0 million upfront fee in connection with the closing of the Luspatercept Agreement;
- \$45.0 million of nonrefundable, upfront license and option payments in connection with the closing of the Original and Amended Sotatercept Agreements;
- \$14.9 million received for sotatercept development and manufacturing activities;
- \$47.9 million received for luspatercept development and manufacturing activities; and
- \$59.5 million milestone payments pursuant to the agreements.

The Company allocated the total transaction price to the identified performance obligations (both satisfied and unsatisfied) using the estimated standalone selling price of each performance obligation as of the adoption date of ASC 606. The Company's estimate of the standalone selling price requires judgment, in particular in estimating the value of the license



rights for luspatercept and sotatercept, which includes assumptions over the projected revenues and expenses, probability of technical and regulatory success and appropriate discount rates.

As of the ASC 606 adoption date, the only remaining undelivered element is participation in the JDC for which there was a deferred revenue balance of \$3.7 million. 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.

As a result of adopting ASC 606 on January 1, 2018, the Company has recorded a cumulative-effect reduction to opening accumulated deficit of \$3.7 million as of January 1, 2018 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. License and milestone revenue for the three months ended March 31, 2018 was zero, as compared to the \$0.1 million that would have been recorded under ASC 605. Deferred revenue as of March 31, 2018 was zero under ASC 606, as compared to a balance of \$3.6 million, which would have resulted under ASC 605.

Through March 31, 2018, under all Celgene arrangements the Company has received net cost-share payments and milestones of \$102.8 million and \$44.4 million for luspatercept and sotatercept, respectively. The Company recorded net cost-sharing revenue of \$3.2 million and \$3.6 million during the three months ended March 31, 2018 and 2017, 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, 2018 and 2017, 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, 2018 and 2017, the Company expensed zero and \$0.1 million, respectively, of milestones and fees, which is recorded as research and development expense.

## 15. 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, 2018 and 2017, respectively, as follows (in thousands):

	Three Months Ended March 31,	
	2018	2017
Research and development	\$ 2,869	\$ 3,662
General and administrative	2,827	2,921
	<u>\$ 5,696</u>	<u>\$ 6,583</u>

In December 2016, the Company entered into a consulting agreement with its former Chief Executive Officer. In accordance with the 2003 Plan and 2013 Plan, any vested shares remain exercisable and any outstanding and unvested options and restricted stock units will continue to vest in accordance with their terms so long as he continues to provide services as a non-employee consultant. During the three months ended March 31, 2018 and 2017, respectively, the Company recognized \$0.3 million and \$1.1 million of stock-based compensation expense within research and development expense related to the agreement.

## 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,	
	2018	2017
Expected volatility	63.0%	65.9%
Expected term (in years)	6.0	6.0
Risk-free interest rate	2.6%	2.2%
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, 2018 (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, 2017	3,452	\$ 29.14	6.72	
Granted	638	\$ 40.77		
Exercised	(359)	\$ 13.15		
Canceled or forfeited	(13)	\$ 39.63		
Outstanding at March 31, 2018	3,718	\$ 32.64	7.61	\$ 26,985
Exercisable at March 31, 2018	1,926	\$ 29.85	6.38	\$ 19,426

- (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, 2018.

During the three months ended March 31, 2018, the Company granted stock options to purchase an aggregate of 637,585 shares of its common stock, with a weighted-average grant date fair value of options granted of \$24.13 per share.

During the three months ended March 31, 2018, current and former employees of the Company exercised a total of 358,685 options, resulting in total proceeds of \$4.7 million.

The aggregate intrinsic value of options exercised during the three months ended March 31, 2018 was \$10.5 million.

As of March 31, 2018, there was \$34.1 million of unrecognized compensation expense related to unvested stock options that is expected to be recognized over a weighted-average period of 2.72 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, 2018 (in thousands, except per share amounts):

	Number of Stock Units	Weighted- Average Grant Date Fair Value Per Share
Unvested balance at December 31, 2017	268	\$ 32.44
Granted	100	\$ 40.63
Vested	(70)	\$ 31.26
Forfeited	—	\$ 40.61
Unvested balance at March 31, 2018	298	\$ 35.47

As of March 31, 2018, there was approximately \$8.9 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. As a result, 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, 2018 (in thousands, except per share amounts):

	Number of Stock Units	Weighted- Average Grant Date Fair Value Per Share
Unvested balance at December 31, 2017	337	\$ 31.53
Granted	—	\$ —
Vested	(4)	\$ 23.44
Forfeited	—	\$ —
Unvested balance at March 31, 2018	333	\$ 31.63

As of March 31, 2018, there was approximately \$3.8 million of related unrecognized compensation cost, which the Company expects to recognize over a remaining weighted-average period of 1.18 years.

**Employee Stock Purchase Plan**

The Company recorded stock-based compensation expense related to the ESPP Plan during the three months ended March 31, 2018 and 2017 of \$0.1 million and \$0.1 million, respectively.

**16. Income Taxes****U.S. Tax Reform**

In March 2018, the FASB issued ASU-2018-05, *Income Taxes (Topic 740), Amendments to SEC Paragraphs Pursuant to SEC Staff Accounting Bulletin No. 118*. This new standard addresses the accounting implications of the Tax Cuts and Jobs Act (the Tax Act) signed into law in December 2017. The Tax Act, among other changes, permanently lowers the corporate federal tax rate to 21% from the existing maximum rate of 35%, effective for tax years beginning January 1, 2018. As a result of the reduction of the corporate federal income tax rate to 21%, US GAAP requires companies to revalue their deferred tax assets and deferred tax liabilities as of the date of enactment, with the resulting tax effects accounted for in the reporting period of the enactment. This revaluation resulted in a provision of \$59.3 million to income tax expense in continuing operations and a corresponding reduction in the valuation allowance as of December 31, 2017. As a result, there was no impact on the Company's consolidated statements of operations from the reduction in the tax rate. The other provisions of the Tax Act did not have a material impact on the consolidated financial statements.

The Company is still in the process of analyzing the impact to the Company of the Tax Act. Where the Company has been able to make reasonable estimates of the effects for which its analysis is not yet complete, the Company has recorded provisional amounts. Where the Company has not yet been able to make reasonable estimates of the impact of certain elements, the Company has not recorded any amounts related to those elements and has continued accounting for them in accordance with ASC 740 on the basis of the tax laws in effect immediately prior to the enactment of the Tax Act.

**Deferred tax assets and valuation allowance**

For the three months ended March 31, 2018 and 2017, the Company recognized income tax expense of \$10 thousand, and \$6 thousand, respectively, related to state income taxes on its interest income.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets as of March 31, 2018 and December 31, 2017.

The Company files income tax returns in the United States, and various state jurisdictions. The federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2014 through December 31, 2017. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state taxing authorities to the extent utilized in a future period.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of March 31, 2018 and December 31, 2017, the Company did not have any significant uncertain tax positions.

## **17. Related Party Transactions**

### **Celgene Corporation**

Celgene owned 12.3% and 12.5% of the Company's fully diluted equity as of March 31, 2018 and December 31, 2017, respectively. Refer to Note 14 for additional information regarding this collaboration arrangement.

During the three months ended March 31, 2018 and 2017, all revenue recognized by the Company was recognized under the Celgene collaboration arrangement and, as of March 31, 2018, 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, 2017.

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, ACE-2494 and our other 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, 2017 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 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. Celgene is currently conducting 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 beta-thalassemia, also known as the "BELIEVE" trial. Celgene has recently initiated a Phase 2 clinical trial in non-transfusion-dependent beta-thalassemia patients, referred to as the "BEYOND" trial. We further expect Celgene to initiate a Phase 3 clinical trial, the "COMMANDS" trial, in first-line, lower-risk MDS patients in the first half of 2018. Enrollment is also currently ongoing in a Phase 2 clinical trial for the treatment of patients with myelofibrosis, a rare bone marrow disorder. If luspatercept were to receive regulatory approval for each of these indications in the United States and Europe, we and Celgene believe that there is an annual peak sales opportunity for luspatercept in excess of \$2 billion across all indications.

For sotatercept, we announced in September 2017 that Celgene granted us rights to fund, develop, and lead the global commercialization of sotatercept in pulmonary hypertension, 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. 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. We expect to initiate a Phase 2 clinical trial for the treatment of patients with PAH in the second quarter of 2018.

For luspatercept and, outside of pulmonary hypertension, 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 pulmonary hypertension, 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 will be entirely funded by Celgene.

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. In January 2018, we announced the preliminary results from the first two cohorts in part 1 of the Phase 2 clinical trial in patients with FSHD showing marked increases in the mean total muscle volume of the muscles treated with ACE-083 measured using magnetic resonance imaging, or MRI. We recently initiated part 2 of the ACE-083 FSHD Phase 2 trial, and we expect to report preliminary results from all dose-escalation cohorts of part 1 in both of our Phase 2 clinical trials with ACE-083 in the second half of this year.

In addition to our mid- to late-stage clinical programs, we initiated a Phase 1 healthy volunteer study in early 2018 with ACE-2494, our wholly owned systemic muscle agent from our proprietary platform technology, IntelliTrap™, and we expect to report initial results from this healthy volunteer study in the first half of 2019. We are also 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, 2018, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$539.7 million from public investors, \$123.7 million in equity investments from our collaboration partners and \$277.3 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, ACE-2494 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. We expect that this will take a number of years and is subject to significant uncertainty. All current and future development and commercialization costs for luspatercept and, outside of pulmonary hypertension, sotatercept, are paid by Celgene. If we obtain regulatory approval for ACE-083, sotatercept, ACE-2494, 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, 2017.

## **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.



[Table of Contents](#)

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, 2018, we have incurred \$578.3 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 pulmonary hypertension, sotatercept, are 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, dalantercept and ACE-083, of which the luspatercept trials are reimbursed by Celgene and the dalantercept trials are 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. Our external research and development expenses during the three months ended March 31, 2018 and 2017 are as follows:

(in thousands)	Three Months Ended March 31,	
	2018	2017
Luspatercept(1)	1,613	1,998
Sotatercept(2)	1,559	—
Dalantercept(3)	41	1,296
ACE-083	2,583	2,028
ACE-2494	598	1,286
Total direct research and development expenses	6,394	6,608
Other expenses(4)	17,037	15,119
Total research and development expenses	\$ 23,431	\$ 21,727

[Table of Contents](#)

- (1) Expenses associated with luspatercept are reimbursed 100% by Celgene.
- (2) These expenses are associated with our pulmonary hypertension activities.
- (3) Development of dalantercept has been discontinued.
- (4) Other expenses include unallocated employee and 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 re-measurement gain or loss associated with the change in the fair value of our common stock warrant liabilities and interest income earned on cash, cash equivalents and investments.

To estimate the fair value of our liability classified warrants, we use 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. We base the estimates in the pricing models, in part, on subjective assumptions, including stock price volatility, risk-free interest rate, dividend yield, and the fair value of the common stock underlying the warrants. The Black-Scholes option pricing model was used at March 31, 2018.

**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, accrued expenses and stock-based compensation. We also utilize significant estimates and assumptions in determining the fair value of our liability-classified warrants to purchase common stock. 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.

Effective January 1, 2018, we adopted ASC Topic 606, the impact of which is discussed further in Note 2 to the financial statements in this Quarterly Report on Form 10-Q. There have been no material changes to our critical accounting policies since December 31, 2017. For further information on our critical and other significant accounting policies, 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, 2017.

## Results of Operations

### Comparison of the Three Months Ended March 31, 2018 and 2017

(in thousands)	Three Months Ended March 31,		Increase (Decrease)
	2018	2017	
Revenue:			
Collaboration revenue:			
License and milestone	\$ —	\$ 135	\$ (135)
Cost-sharing, net	3,232	3,570	(338)
Total revenue	3,232	3,705	(473)
Costs and expenses:			
Research and development	23,431	21,727	1,704
General and administrative	7,441	7,836	(395)
Total costs and expenses	30,872	29,563	1,309
Loss from operations	(27,640)	(25,858)	(1,782)
Other income, net	1,431	457	974
Loss before income taxes	(26,209)	(25,401)	(808)
Income tax provision	(10)	(6)	(4)
Net loss	\$ (26,219)	\$ (25,407)	\$ (812)

**Revenue.** We recognized revenue of \$3.2 million in the three months ended March 31, 2018, compared to \$3.7 million in the same period in 2017. All of the revenue in both periods was derived from the Celgene agreements. Significant factors resulting in this \$0.5 million decrease include:

- a decrease in license and milestone revenue of \$0.1 million resulting from the adoption of Accounting Standards Codification Topic 606, *Revenue from Contracts with Customers*, (ASC 606), the impact of which is discussed further in Note 11 to the financial statements in this Quarterly Report on Form 10-Q; and
- a decrease in cost-sharing revenue of \$0.3 million primarily due to the timing of our activities related to the luspatercept Phase 2 clinical trials.

**Research and Development Expenses.** Research and development expenses were \$23.4 million in the three months ended March 31, 2018, compared to \$21.7 million in the same period in 2017. Significant factors resulting in this \$1.7 million increase include:

- an increase in personnel expenses totaling \$2.8 million, primarily related to increased headcount supporting the growth of our wholly owned therapeutic candidates and preclinical programs; and
- a decrease in toxicology expenses of \$1.5 million due to a reduction in the work related to ongoing toxicology studies.

**General and Administrative Expenses.** General and administrative expenses were \$7.4 million in the three months ended March 31, 2018, compared to \$7.8 million for the same period in 2017. Significant factors resulting in this \$0.4 million decrease include:

- a decrease in consulting expenses totaling \$0.7 million, from the comparable period in 2017; and
- an increase in professional fees of \$0.2 million due to an increase in audit, legal, regulatory, and tax-related services associated with being a public company.

**Other Income, Net.** Other income, net was \$1.4 million in the three months ended March 31, 2018, compared to \$0.5 million for the same period in 2017. This \$0.9 million change was primarily due to a \$0.8 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, 2018, we had an accumulated deficit of \$495.5 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.

As of March 31, 2018, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$539.7 million from public investors, \$123.7 million in equity investments from our collaboration partners and \$277.3 million in upfront payments, milestones, and net research and development payments from our collaboration partners.

As of March 31, 2018, we had \$353.3 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. We believe that our existing cash, cash equivalents and investments will be sufficient to fund our projected operating requirements into 2021.

### 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,	
	2018	2017
Net cash (used in) provided by:		
Operating activities	\$ (23,431)	\$ (21,763)
Investing activities	(11,921)	32,635
Financing activities	4,938	1,521
Net (decrease) increase in cash, cash equivalents and restricted cash	\$ (30,414)	\$ 12,393

#### *Operating Activities*

Net cash used in operating activities was \$23.4 million for the three months ended March 31, 2018 compared to \$21.8 million during the same period in 2017. The increase in cash used in operating activities of \$1.6 million was driven primarily by a change in accrued expenses.

#### *Investing Activities*

Net cash used in investing activities was \$11.9 million for the three months ended March 31, 2018 compared to net cash provided by investing activities of \$32.6 million during the same period in 2017. This decrease in cash used in investing activities of \$44.6 million was primarily due to net purchases of our investments of \$11.4 million during the three months ended March 31, 2018 as we invested the the money we raised in our September 2017 public offering pursuant to our investment policy, compared to net maturities of \$33.3 million in the three months ended March 31, 2017, in connection with managing our investment portfolio to meet our projected cash requirements.

#### *Financing Activities*

Net cash provided by financing activities was \$4.9 million for the three months ended March 31, 2018 compared to \$1.5 million for the same period in 2017. The increase in cash provided by financing activities of \$3.4 million is primarily attributable to an increase of \$3.6 million in cash proceeds from the exercise of stock options.

### 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, ACE-2494 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.

We believe that our existing cash, cash equivalents and investments will be sufficient to fund our projected operating requirements into 2021. However, we will require additional capital for the further development of our existing therapeutic candidates and may also need to raise additional funds sooner to pursue other development activities related to additional therapeutic candidates.

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 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 \$145.7 million as of December 31, 2017, 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, 2017, we had federal NOL carryforwards of approximately \$438.0 million and state NOL carryforwards of \$393.6 million available to reduce future taxable income, if any. These federal and state NOL carryforwards expire at various times through 2037. 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, 2017.

### **Contractual Obligations and Commitments**

During the three months ended March 31, 2018, 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, 2017.

### **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, 2018 and December 31, 2017, we had cash, cash equivalents and investments of \$353.3 million and \$372.9 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, 2018, 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, 2018, 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, 2018, we implemented certain internal controls in connection with our adoption of ASC Topic 606. 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, 2017.

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

#### (a) Unregistered Sales of Equity Securities

We issued the following unregistered securities during the three months ended March 31, 2018:

- In January 2018, we issued 18,449 shares of Common Stock upon the cashless exercise of warrants to purchase 21,258 shares of Common Stock.

These issuances of shares of our Common Stock were exempt from registration under the Securities Act of 1933, or the Securities Act, pursuant to Rule 506 of Regulation D of the Securities Act and Section 4(a)(2) of the Securities Act.

### Item 6. Exhibits

Exhibit Number	Description of Exhibit
10.1*	<a href="#">Separation and Transition Agreement, by and between Matthew L. Sherman and Acceleron Pharma Inc., dated as of January 2, 2018 (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36065), filed on January 3, 2018)</a>
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

\* Management contract or compensatory plan or arrangement.



**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 8, 2018

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

Date: May 8, 2018

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 8, 2018

/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 8, 2018

/s/ Kevin F. McLaughlin

Date

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, 2018 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 8, 2018

By: /s/ Habib J. Dable

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

Date: May 8, 2018

By: /s/ Kevin F. McLaughlin

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

