

ACCELERON PHARMA INC

Form 10-Q

August 03, 2017

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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 June 30, 2017

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 2836 27-0072226

(State or other jurisdiction of (Primary Standard Industrial (I.R.S. Employer  
incorporation or organization) Classification Code Number) 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

Accelerated filer

Non-accelerated filer  (Do not check if a smaller reporting company) Smaller reporting company   
Emerging growth company

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

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

As of July 31, 2017, there were 38,685,073 shares of the registrant's Common Stock, par value \$0.001 per share, outstanding.

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## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements

Acceleron Pharma Inc.

Condensed Consolidated Balance Sheets

(amounts in thousands except share and per share data)

(unaudited)

|  | June 30,<br>2017 | December 31,<br>2016 |
|--|------------------|----------------------|
| Assets   |                  |                      |
| Current assets:  |                  |                      |
| Cash and cash equivalents  | \$ 30,726        | \$ 20,950            |
| Collaboration receivables (all amounts are with related party)   | 2,881            | 3,234                |
| Prepaid expenses and other current assets  | 3,111            | 3,862                |
| Short-term investments   | 111,695          | 118,740              |
| Total current assets   | 148,413          | 146,786              |
| Property and equipment, net  | 7,210            | 5,201                |
| Restricted cash  | 1,132            | 946                  |
| Other assets   | 91               | 22                   |
| Long-term investments  | 51,617           | 94,692               |
| Total assets   | \$ 208,463       | \$ 247,647           |
| Liabilities and stockholders' equity   |                  |                      |
| Current liabilities:   |                  |                      |
| Accounts payable   | \$ 1,661         | \$ 1,590             |
| Accrued expenses   | 11,370           | 13,249               |
| Deferred revenue   | 541              | 541                  |
| Deferred rent  | 727              | 769                  |
| Total current liabilities  | 14,299           | 16,149               |
| Deferred revenue, net of current portion   | 3,432            | 3,704                |
| Deferred rent, net of current portion  | 684              | 953                  |
| Warrants to purchase common stock  | 1,516            | 1,244                |
| Total liabilities  | 19,931           | 22,050               |
| Commitments and contingencies (Note 14)  |                  |                      |
| Stockholders' equity:  |                  |                      |
| Undesignated preferred stock, \$0.001 par value: 25,000,000 shares authorized and no shares issued or outstanding  | —                | —                    |
| Common stock, \$0.001 par value: 175,000,000 shares authorized; 38,636,505 and 38,251,826 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively | 39               | 39                   |
| Additional paid-in capital   | 608,493          | 590,474              |
| Accumulated deficit  | (419,646 )       | (364,491 )           |
| Accumulated other comprehensive loss   | (354 )           | (425 )               |
| Total stockholders' equity   | 188,532          | 225,597              |
| Total liabilities and stockholders' equity   | \$ 208,463       | \$ 247,647           |

See accompanying notes to these condensed consolidated financial statements.



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Acceleron Pharma Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(amounts in thousands except per share data)

(unaudited)

|   | Three Months Ended<br>June 30, |            | Six Months Ended<br>June 30, |            |
|---|--------------------------------|------------|------------------------------|------------|
|   | 2017                           | 2016       | 2017                         | 2016       |
| Revenue:  |                                |            |                              |            |
| Collaboration revenue:  |                                |            |                              |            |
| License and milestone   | \$ 135                         | \$ 135     | \$ 271                       | \$ 15,279  |
| Cost-sharing, net   | 2,922                          | 3,060      | 6,491                        | 6,117      |
| Total revenue (all amounts are with related party)  | 3,057                          | 3,195      | 6,762                        | 21,396     |
| Costs and expenses:   |                                |            |                              |            |
| Research and development  | 21,598                         | 16,138     | 43,327                       | 32,390     |
| General and administrative  | 11,370                         | 6,712      | 19,203                       | 12,618     |
| Total costs and expenses  | 32,968                         | 22,850     | 62,530                       | 45,008     |
| Loss from operations  | (29,911 )                      | (19,655 )  | (55,768 )                    | (23,612 )  |
| Other (expense) income, net   | (228 )                         | (2,864 )   | (272 )                       | 5,819      |
| Interest income   | 476                            | 503        | 977                          | 837        |
| Total other income (expense) net  | 248                            | (2,361 )   | 705                          | 6,656      |
| Loss before income taxes  | (29,663 )                      | (22,016 )  | (55,063 )                    | (16,956 )  |
| Income tax provision  | (6 )                           | —          | (12 )                        | —          |
| Net loss applicable to common stockholders  | \$(29,669)                     | \$(22,016) | \$(55,075)                   | \$(16,956) |
| Net loss per share applicable to common stockholders-basic and diluted<br>(Note 9)                                  | \$(0.77 )                      | \$(0.59 )  | \$(1.43 )                    | \$(0.46 )  |
| Weighted-average number of common shares used in computing net loss<br>per share applicable to common stockholders: | 38,631                         | 37,272     | 38,515                       | 37,092     |
| Other comprehensive loss:   |                                |            |                              |            |
| Net loss  | \$(29,669)                     | \$(22,016) | \$(55,075)                   | \$(16,956) |
| Net unrealized holding gains on short-term and long-term investments<br>during the period                           | 46                             | 227        | 71                           | 472        |
| Comprehensive loss  | \$(29,623)                     | \$(21,789) | \$(55,004)                   | \$(16,484) |

See accompanying notes to these condensed consolidated financial statements.

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Acceleron Pharma Inc.

Condensed Consolidated Statements of Cash Flows

(amounts in thousands)

(unaudited)

|  | Six Months Ended<br>June 30, |            |
|--|------------------------------|------------|
|  | 2017                         | 2016       |
| Operating Activities   |                              |            |
| Net loss   | \$(55,075)                   | \$(16,956) |
| Adjustments to reconcile net loss to net cash used in operating activities:          |                              |            |
| Depreciation and amortization  | 1,224                        | 715        |
| Loss on disposition of fixed assets  | —                            | 19         |
| Stock-based compensation   | 16,172                       | 8,800      |
| Change in fair value of warrants   | 272                          | (5,819 )   |
| Net amortization of premium on investments   | 145                          | (432 )     |
| Changes in assets and liabilities:   |                              |            |
| Prepaid expenses and other assets  | 682                          | (619 )     |
| Collaboration receivables  | 353                          | 389        |
| Accounts payable   | 71                           | (309 )     |
| Accrued expenses   | (2,185 )                     | (525 )     |
| Restricted cash  | (186 )                       | (200 )     |
| Deferred revenue   | (272 )                       | (280 )     |
| Deferred rent  | (311 )                       | 284        |
| Net cash used in operating activities  | (39,110 )                    | (14,933 )  |
| Investing Activities   |                              |            |
| Purchases of investments   | (175 )                       | (160,798 ) |
| Proceeds from sales and maturities of investments                                    | 50,221                       | 44,178     |
| Purchases of property and equipment  | (2,928 )                     | (1,560 )   |
| Net cash provided by (used in) investing activities                                  | 47,118                       | (118,180)  |
| Financing Activities   |                              |            |
| Proceeds from issuance of common stock from public offering, net issuance costs      | —                            | 140,391    |
| Payments for withholding taxes on restricted stock units                             | (140 )                       | —          |
| Proceeds from exercise of stock options and warrants to purchase common stock        | 1,398                        | 1,550      |
| Proceeds from issuances of common stock related to employee stock purchase plan      | 510                          | 384        |
| Net cash provided by financing activities  | 1,768                        | 142,325    |
| Net increase in cash and cash equivalents  | 9,776                        | 9,212      |
| Cash and cash equivalents at beginning of period                                     | 20,950                       | 27,783     |
| Cash and cash equivalents at end of period   | \$30,726                     | \$36,995   |
| Supplemental Disclosure of Non-Cash Investing and Financing Activities:              |                              |            |
| Purchase of property and equipment included in accounts payable and accrued expenses | \$305                        | \$258      |

See accompanying notes to these condensed consolidated financial statements.

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Acceleron Pharma Inc.

Notes to Condensed Consolidated Financial Statements  
(unaudited)

1. Nature of Business

Acceleron Pharma Inc. (Acceleron or the Company) is a Cambridge, Massachusetts-based clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases. The Company's research focuses on key natural regulators of cellular growth and repair, particularly the Transforming Growth Factor-Beta (TGF-beta) protein superfamily. By combining its discovery and development expertise, including its proprietary knowledge of the TGF-beta superfamily, and its internal protein engineering and manufacturing capabilities, the Company has built a highly productive discovery and development platform that has generated innovative therapeutic candidates with novel mechanisms of action.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, 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, 2016, 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 June 30, 2017, the results of its operations for the three and six months ended June 30, 2017 and 2016, and its cash flows for the six months ended June 30, 2017 and 2016.

The results for the three and six months ended June 30, 2017 are not necessarily indicative of the results to be expected for the year ending December 31, 2017, 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, 2016, 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, 2016.

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 June 30, 2017, 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, 2016, have not changed, except for the adoption of Accounting Standards Update (ASU) No. 2016-09, Compensation - Stock Compensation (Topic 718), Improvements to Employee Share-Based Payment Accounting, which is discussed further in Note 16.

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

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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, accrued expenses, and the recoverability of the Company's net deferred tax assets and related valuation allowance.

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. All material long-lived assets of the Company reside in the United States. The Company does use contract research organizations (CROs) and research institutions located outside the United States. Some of these expenses are subject to collaboration reimbursement which is presented as a component of cost sharing, net in the consolidated statements of operations and comprehensive (loss) income.

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 June 30, 2017 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 and six months ended June 30, 2017 and 2016.

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 June 30, 2017 and December 31, 2016 was \$127.5 million and \$172.2 million, respectively. The aggregate fair value of securities held by the Company in an unrealized loss position for more than twelve months as of June 30, 2017 and December 31, 2016 was \$17.1 million and \$5.5 million, respectively. The aggregate unrealized loss for those securities in an unrealized loss position for more than twelve months is \$0.1 million and \$2 thousand, respectively. As a result, the Company determined it did not hold any investments with any other-than-temporary impairment as of June 30, 2017 and December 31, 2016.



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The following is a summary of cash, cash equivalents and available-for-sale securities as of June 30, 2017 and December 31, 2016 (in thousands):

|  | June 30, 2017     |                              |                               |                            |
|--|-------------------|------------------------------|-------------------------------|----------------------------|
|  | Amortized<br>Cost | Gross<br>Unrealized<br>Gains | Gross<br>Unrealized<br>Losses | Estimated<br>Fair<br>Value |
| Cash and cash equivalents due in 90 days or less                     | \$30,726          | \$ —                         | \$ —                          | \$30,726                   |
| Available-for-sale securities:                                       |                   |                              |                               |                            |
| Corporate obligations due in one year or less                        | 48,762            | 1                            | (69 )                         | 48,694                     |
| Corporate obligations due in more than one year                      | 17,479            | —                            | (69 )                         | 17,410                     |
| U.S. Treasury securities due in one year or less                     | 14,998            | —                            | (10 )                         | 14,988                     |
| U.S. Treasury securities due in more than one year                   | 11,530            | —                            | (63 )                         | 11,467                     |
| Certificates of deposit due in one year or less                      | 8,152             | —                            | —                             | 8,152                      |
| Certificates of deposit due in more than one year                    | 4,309             | —                            | —                             | 4,309                      |
| Mortgage and other asset backed securities due in one year or less   | 39,905            | —                            | (43 )                         | 39,862                     |
| Mortgage and other asset backed securities due in more than one year | 18,530            | —                            | (100 )                        | 18,430                     |
| Total available-for-sale securities                                  | \$163,665         | \$ 1                         | \$ (354 )                     | \$163,312                  |
| Total cash, cash equivalents and available-for-sale securities       | \$194,391         | \$ 1                         | \$ (354 )                     | \$194,038                  |
|  | December 31, 2016 |                              |                               |                            |
|  | Amortized<br>Cost | Gross<br>Unrealized<br>Gains | Gross<br>Unrealized<br>Losses | Estimated<br>Fair<br>Value |
| Cash and cash equivalents due in 90 days or less                     | \$20,950          | \$ —                         | \$ —                          | \$20,950                   |
| Available-for-sale securities:                                       |                   |                              |                               |                            |
| Corporate obligations due in one year or less                        | 45,839            | 1                            | (58 )                         | 45,782                     |
| Corporate obligations due in more than one year                      | 42,895            | —                            | (185 )                        | 42,710                     |
| U.S. Treasury securities due in one year or less                     | 22,490            | —                            | (10 )                         | 22,480                     |
| U.S. Treasury securities due in more than one year                   | 11,541            | —                            | (53 )                         | 11,488                     |
| Certificates of deposit due in one year or less                      | 13,562            | —                            | —                             | 13,562                     |
| Certificates of deposit due in more than one year                    | 9,811             | —                            | —                             | 9,811                      |
| Mortgage and other asset backed securities due in one year or less   | 36,948            | —                            | (32 )                         | 36,916                     |
| Mortgage and other asset backed securities due in more than one year | 30,771            | —                            | (88 )                         | 30,683                     |
| Total available-for-sale securities                                  | \$213,857         | \$ 1                         | \$ (426 )                     | \$213,432                  |
| Total cash, cash equivalents and available-for-sale securities       | \$234,807         | \$ 1                         | \$ (426 )                     | \$234,382                  |

## 6. Restricted Cash

As of June 30, 2017 and December 31, 2016, the Company maintained letters of credit totaling \$1.1 million and \$0.9 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



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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 June 30, 2017 and December 31, 2016 (in thousands):

|  | June 30, 2017   |  |  | Total            |
|--|---|--|--|------------------|
|  | Quoted Prices<br>in Active Markets<br>for Identifiable<br>(Level 1) | Significant<br>Observable<br>Inputs<br>(Level 2) | Significant<br>Unobservable<br>Inputs<br>(Level 3) |                  |
| <b>Assets:</b>                             |   |  |  |                  |
| Money market funds                         | \$26,230  | \$ —   | \$ —   | \$26,230         |
| Corporate obligations                      | —   | 66,104   | —  | 66,104           |
| U.S. Treasury securities                   | —   | 26,455   | —  | 26,455           |
| Certificates of deposit                    | —   | 12,462   | —  | 12,462           |
| Mortgage and other asset backed securities | —   | 58,292   | —  | 58,292           |
| Restricted cash                            | 1,132   | —  | —  | 1,132            |
| <b>Total assets</b>                        | <b>\$27,362</b>   | <b>\$ 163,313</b>                                | <b>\$ —</b>  | <b>\$190,675</b> |
| <b>Liabilities:</b>                        |   |  |  |                  |
| Warrants to purchase common stock          | \$—   | \$ —   | \$ 1,516   | \$1,516          |
| <b>Total liabilities</b>                   | <b>\$—</b>  | <b>\$ —</b>                                      | <b>\$ 1,516</b>                                    | <b>1,516</b>     |
| <br>                                       |   |  |  |                  |
|  | December 31, 2016   |  |  | Total            |
|  | Quoted Prices<br>in Active Markets<br>for Identifiable<br>(Level 1) | Significant<br>Observable<br>Inputs<br>(Level 2) | Significant<br>Unobservable<br>Inputs<br>(Level 3) |                  |
| <b>Assets:</b>                             |   |  |  |                  |
| Money market funds                         | \$19,818  | \$ —   | \$ —   | \$19,818         |
| Corporate obligations                      | —   | 88,492   | —  | 88,492           |
| U.S. Treasury securities                   | —   | 33,968   | —  | 33,968           |
| Certificates of deposit                    | —   | 23,373   | —  | 23,373           |
| Mortgage and other asset backed securities | —   | 67,599   | —  | 67,599           |
| Restricted cash                            | 946   | —  | —  | 946              |
| <b>Total assets</b>                        | <b>\$20,764</b>   | <b>\$ 213,432</b>                                | <b>\$ —</b>  | <b>\$234,196</b> |
| <b>Liabilities:</b>                        |   |  |  |                  |
| Warrants to purchase common stock          | \$—   | \$ —   | \$ 1,244   | \$1,244          |
| <b>Total liabilities</b>                   | <b>\$—</b>  | <b>\$ —</b>                                      | <b>\$ 1,244</b>                                    | <b>\$1,244</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 six months ended June 30, 2017 or the year ended December 31, 2016.

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 13). During the periods presented, the Company has not changed the manner in

which it values assets and liabilities that are measured at fair value using Level 3 inputs.

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

|                      | Six Months<br>Ended June 30, |          |
|----------------------|------------------------------|----------|
|                      | 2017                         | 2016     |
| Beginning balance    | \$1,244                      | \$17,187 |
| Change in fair value | 272                          | (5,819 ) |
| Ending balance       | \$1,516                      | \$11,368 |

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 June 30, 2017, 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 six months ended June 30, 2017 or the year ended December 31, 2016.

#### 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<br>Months<br>Ended<br>June 30, |       | Six Months<br>Ended<br>June 30, |       |
|--|--------------------------------------|-------|---------------------------------|-------|
|  | 2017                                 | 2016  | 2017                            | 2016  |
| Outstanding stock options                          | 3,541                                | 3,503 | 3,541                           | 3,503 |
| Common stock warrants                              | 64                                   | 397   | 64                              | 397   |
| Shares issuable under employee stock purchase plan | 13                                   | 13    | 13                              | 13    |
| Restricted stock units                             | 810                                  | 608   | 810                             | 608   |
|  | 4,428                                | 4,521 | 4,428                           | 4,521 |

#### 10. Comprehensive (Loss) Income

Comprehensive (loss) income 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) income is presented separately on the consolidated balance sheets and consists entirely of unrealized holding gains on investments as of June 30, 2017 and December 31, 2016.

#### 11. Subsequent Events

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 relative to certain estimates or to identify matters that require additional disclosure. The Company has evaluated all subsequent events and determined that there are no material recognized or unrecognized subsequent events requiring disclosure except as described below.

On July 18, 2017, the Company entered into a five-year lease extension for approximately 37,700 square feet of office, manufacturing and lab space located at 128 Sidney Street, Cambridge Massachusetts, our principal headquarters and manufacturing facility. The lease commences on October 1, 2018 and ends on September 30, 2023.



The total operating lease obligation for the term of this agreement, excluding operating costs and real estate taxes, is approximately \$13.0 million. The Company has the option to extend the lease by an additional five years.

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On July 18, 2017, the Company entered into a five-year lease extension for approximately 37,116 square feet of office and lab space located at 149 Sidney Street, Cambridge Massachusetts. The lease commences on October 1, 2018 and ends on September 30, 2023. The total operating lease obligation for the term of this agreement, excluding operating costs and real estate taxes, is approximately \$12.8 million. The Company has the option to extend the lease by an additional five years.

The leases provide for contributions from the landlords of up to \$0.7 million for certain work to be performed by the Company and for the replacement of certain HVAC units, water heaters and other base building improvements at both 128 Sidney Street and 149 Sidney Street.

On July 27, 2017, the Company entered into a one-year sublease with Rubius Therapeutics, Inc. (the "sublessee") under which Rubius will sublease from us approximately 11,825 square feet of office space and lab space at 99 Erie Street, Cambridge, Massachusetts (the "sublease"). The sublease commenced on August 1, 2017 and ends on July 31, 2018. The total operating lease obligation, excluding operating costs and real estate taxes, for the term of this agreement is approximately \$0.7 million. The sublessee has the right to extend the sublease by an additional five months.

### 12. 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 May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. The new standard will be effective for the Company on January 1, 2018. Topic 606 allows for either a full retrospective application, in which the standard is applied to all periods presented, or a modified retrospective application, in which the standard is applied to the most current period presented in the financial statements. As of June 30, 2017, revenue is generated exclusively from the Company's collaboration agreement with Celgene. The Company is currently evaluating the potential impact that Topic 606 may have on its financial position and results of operations as it relates to this single arrangement, and expects to elect the modified retrospective application as its transition method.

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 November 2016, the FASB issued 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. This guidance is effective for annual and interim reporting periods beginning after December 15, 2017, and requires retrospective application. The Company is currently assessing the impact that adopting ASU 2016-18 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

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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.

## 13. 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 |                   |
|-----------------------------------|----------------|-------------------|---|------------------------------|------------------------------|-------------------|
|                                   | June 30, 2017  | December 31, 2016 |   |                              | June 30, 2017                | December 31, 2016 |
| Warrants to purchase common stock | 61             | 61                | \$ 5.88                                   | June 10, 2020 - July 9, 2020 | Liability                    | Liability         |
| Warrants to purchase common stock | 3              | 4                 | 7.40                                      | December 31, 2017            | Equity(1) (2)                | Equity(1) (2)     |
| All warrants                      | 64             | 65                | \$ 5.94                                   |                              |                              |                   |

(1) In March 2017, warrant holders exercised warrants to purchase 1,187 shares of Common Stock on a net basis, resulting in the issuance of 1,014 shares of Common Stock.

(2) Warrants to purchase common stock were issued in connection with various debt financing transactions that were consummated in periods prior to our initial public offering.

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 income (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 an increase in fair value of \$0.2 million and \$2.9 million for the three months ended June 30, 2017 and 2016, respectively, and an increase in fair value of \$0.3 million and a decrease of \$5.8 million for the six months ended June 30, 2017 and 2016, respectively, which was recorded in other (expense) income 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 June 30, 2017 and December 31, 2016.

## 14. Commitments and Contingencies

## Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of its business. The Company was not subject to any material legal proceedings during the three months ended June 30, 2017, and, to the best of its knowledge, no material legal proceedings are currently pending or threatened.

99 Erie Street Operating Lease

On March 16, 2017, the Company entered into an approximately six and a half year lease for approximately 11,825 square feet of rentable office and lab space located at 99 Erie Street, Cambridge, Massachusetts. The lease commenced on May 1, 2017 and ends on September 30, 2023. Excluding operating costs and real estate taxes, rent for the first year is \$0.7 million with annual rent escalations thereafter. The total operating lease obligation for the term of this agreement, excluding operating

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costs and real estate taxes, is \$5.1 million. In addition, the lease provides for an optional contribution from the landlord towards the build-out of the space of up to \$0.1 million. The Company has the option to extend the lease by an additional three years. In accordance with the lease, the Company entered into a cash-collateralized, irrevocable standby letter of credit in the amount of \$0.2 million, naming the landlord as beneficiary.

Other

The Company is also party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at June 30, 2017 and December 31, 2016, or royalties on future sales of specified products. No milestones or royalty payments under these agreements are expected to be payable in the immediate future. See Note 15 for discussion of these arrangements.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company indemnifies, holds harmless, and agrees to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners or customers, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third party with respect to the Company's products. The term of these indemnification agreements is generally perpetual any time after execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

15. Significant Agreements

Celgene

Overview

On February 20, 2008 the Company entered into a collaboration, license, and option agreement with Celgene Corporation (Celgene) relating to sotatercept (the Sotatercept Agreement). On August 2, 2011, the Company entered into a second collaboration, license and option agreement with Celgene for luspatercept (the Luspatercept Agreement), and also amended certain terms of the Sotatercept Agreement. These agreements provide Celgene exclusive licenses for sotatercept and luspatercept in all indications, as well as exclusive rights to obtain a license to certain future compounds.

There have been no material changes to the key terms of the Sotatercept and Luspatercept Agreements since December 31, 2016. For further information on the terms of the agreements as well as the historical accounting analysis, please see the notes to the consolidated financial statements included in the Company's Form 10-K for the year ended December 31, 2016.

Sotatercept Agreement

Under the terms of the Sotatercept Agreement, the Company and Celgene collaborate worldwide for the joint development and commercialization of sotatercept. The Company also granted Celgene an option to license three discovery stage compounds. Under the terms of the agreement, the Company and Celgene jointly develop, manufacture and commercialize sotatercept.

The Company retained responsibility for research and development 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. Celgene will be responsible for any Phase 3 clinical trials, as well as additional Phase 2 clinical trials, and will be responsible for overseeing the manufacture of Phase 3 and commercial supplies by third party contract manufacturing organizations.

Through June 30, 2017, the Company has received \$44.0 million in research and development funding and milestone payments for sotatercept under the original and modified agreements. The next likely clinical milestone payment would be \$10.0 million and result from Celgene's start of a Phase 3 study.

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### 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 has not yet identified additional compounds for the treatment of anemia. Accordingly, there is no assurance that the Company will generate future value from additional programs.

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

Through June 30, 2017, the Company has received \$93.8 million in research and development funding and milestone payments for luspatercept. The next likely milestone payment 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.

### Both Agreements

The Company and Celgene shared development costs under the Sotatercept and Luspatercept Agreements through December 31, 2012. Since January 1, 2013, Celgene has been responsible for paying 100% of worldwide development costs under both agreements. Celgene will be responsible for all commercialization costs worldwide. The Company has the right to co-promote sotatercept, luspatercept and future products under both agreements in North America. The Company will receive tiered royalties in the low-to-mid 20% range on net sales of sotatercept and luspatercept. The royalty schedules for sotatercept and luspatercept are the same.

### Accounting Analysis

During the three months ended June 30, 2017 and 2016, the Company recognized \$0.1 million and \$0.1 million, respectively, and during the six months ended June 30, 2017 and 2016, \$0.3 million and \$0.3 million, respectively, of the total deferred revenue as license and milestone revenue in the accompanying consolidated statements of operations and comprehensive loss.

As noted above, under the terms of the Luspatercept Agreement the Company retained responsibility for certain research and development activities. In November 2013, the Company agreed to conduct additional activities for the benefit of the luspatercept program including certain clinical and non-clinical services. These activities are reimbursed under the same terms and rates of the existing Agreements and are accounted for as the services are delivered.

Pursuant to the terms of the agreement, Celgene and the Company shared development costs, with Celgene responsible for substantially more than half of the costs for sotatercept and luspatercept until December 31, 2012 and 100% of the costs from January 1, 2013 and thereafter. Payments from Celgene with respect to research and development costs incurred by the Company are recorded as cost-sharing revenue. The Company recorded net cost-sharing revenue of \$2.9 million and \$3.1 million during the three months ended June 30, 2017 and 2016, respectively, and \$6.5 million and \$6.1 million during the six months ended June 30, 2017 and 2016, 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 a royalty ranging from 1.0%-3.5% of net sales on any products under the licenses. During the three months ended June 30, 2017 and 2016, the Company expensed \$0.1 million and \$0.1 million, respectively, and during the six months ended June 30, 2017

and 2016, respectively, the Company expensed \$0.1 million and \$1.0 million of milestones and fees defined under the agreement.



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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 June 30, 2017 and 2016, the Company expensed \$0.1 million and \$0.2 million, respectively, and during the six months ended June 30, 2017 and 2016, the Company expensed \$0.2 million and \$0.5 million, respectively, of milestones and fees, which is recorded as research and development expense.

## 16. Stock-Based Compensation

The Company recognized stock-based compensation expense related to stock options, restricted stock units and the 2013 Employee Stock Purchase Plan totaling \$9.6 million, \$4.6 million, \$16.2 million, and \$8.8 million during the three months ended June 30, 2017 and 2016 and the six months ended June 30, 2017 and 2016, respectively.

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

|                            | Three Months Ended |         | Six Months Ended |         |
|----------------------------|--------------------|---------|------------------|---------|
|                            | June 30,           |         | June 30,         |         |
|                            | 2017               | 2016    | 2017             | 2016    |
| Research and development   | \$3,433            | \$1,850 | \$7,095          | \$3,676 |
| General and administrative | 6,155              | 2,701   | 9,077            | 5,124   |
|                            | \$9,588            | \$4,551 | \$16,172         | \$8,800 |

On January 1, 2017, the Company adopted ASU 2016-09, which identifies areas for simplification involving several aspects of accounting for share-based payments, including income tax consequences, classification of awards as either equity or liabilities, an option to make a policy election to recognize gross share based compensation expense with actual forfeitures recognized as they occur as well as certain classification changes on the statement of cash flows. In connection with the adoption of this standard, the Company changed its accounting policy to record actual forfeitures as they occur, rather than estimating forfeitures by applying a forfeiture rate. The provisions of the standard related to the timing of when excess tax benefits are recognized, minimum statutory withholding requirements and forfeitures were adopted using a modified retrospective transition method. Accordingly, a cumulative adjustment of \$0.1 million was booked to retained earnings for the impact of the forfeitures. The Company also recorded \$21.5 million for the excess tax benefit related to equity awards, which was offset by a \$21.5 million increase to the valuation allowance. The provisions of the standard related to the recognition of the excess tax benefits in the income statement and classification in the statement of cash flows were adopted prospectively and prior periods were not retrospectively adjusted.

In December 2016, the Company entered into a consulting agreement with the Company's former Chief Executive Officer. In accordance with the Company's Plan, any vested stock options (options) remain outstanding and exercisable 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 and six months ended June 30, 2017, the Company recognized \$1.0 million and \$2.2 million of stock-based compensation expense within research and development expense associated with these awards.

In April 2017, the Company amended the employment agreement with its Chief Operating Officer as a result of his diagnosis of amyotrophic lateral sclerosis (ALS). The amended agreement modified the vesting conditions of his stock options (options) and restricted stock units in the event his termination of employment as a result of death or disability and extended the post termination exercise period of the options. These modifications resulted in \$3.6 million of stock-based compensation, recognized within general and administrative expense during the second quarter of 2017.

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## Stock Options

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

|                          | Three Months Ended |       | Six Months Ended |       |
|--------------------------|--------------------|-------|------------------|-------|
|                          | June 30,           |       | June 30,         |       |
|                          | 2017               | 2016  | 2017             | 2016  |
| Expected volatility      | 65.8%              | 64.5% | 65.9%            | 64.4% |
| Expected term (in years) | 6.0                | 6.0   | 6.0              | 5.9   |
| Risk-free interest rate  | 1.9 %              | 1.5 % | 2.2 %            | 1.4 % |
| Expected dividend yield  | — %                | — %   | — %              | — %   |

The following table summarizes the stock option activity under the Company's 2003 Stock Option and Restricted Stock Plan and 2013 Equity Incentive Plan during the six months ended June 30, 2017 (in thousands, except per share amounts and years):

|                                  | Number of Grants | Weighted-Average Exercise Price Per Share | Weighted-Average Contractual Life (in years) | Aggregate Intrinsic Value(1) |
|----------------------------------|------------------|---|--|------------------------------|
| Outstanding at December 31, 2016 | 3,316            | \$ 25.96                                  | 7.03   |                              |
| Granted                          | 593              | \$ 29.87                                  |  |                              |
| Exercised                        | (333 )           | \$ 4.20                                   |  |                              |
| Canceled or forfeited            | (35 )            | \$ 35.31                                  |  |                              |
| Outstanding at June 30, 2017     | 3,541            | \$ 28.57                                  | 7.40   | \$ 17,917                    |
| Exercisable at June 30, 2017     | 1,778            | \$ 24.49                                  | 5.93   | \$ 16,464                    |

(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 June 30, 2017.

During the six months ended June 30, 2017, the Company granted stock options to purchase an aggregate of 593,409 shares of its common stock, with a weighted-average grant date fair value of options granted of \$18.08 per share.

During the six months ended June 30, 2017, current and former employees of the Company exercised a total of 332,980 options, resulting in total proceeds of \$1.4 million.

The aggregate intrinsic value of options exercised during the six months ended June 30, 2017 was \$7.7 million.

As of June 30, 2017, there was \$27.7 million of unrecognized compensation expense related to unvested stock options that is expected to be recognized over a weighted-average period of 2.70 years.

## Restricted Stock Units

The following table summarizes the restricted stock unit (RSU) activity under the 2013 Equity Incentive Plan during the six months ended June 30, 2017 (in thousands, except per share amounts):

|                                       | Number of Grants | Weighted-Average Grant Date Fair Value |
|---------------------------------------|------------------|--|
| Unvested balance at December 31, 2016 | 732              | \$ 31.55                               |
| Granted                               | 121              | \$ 29.20                               |
| Vested                                | (35 )            | \$ 30.84                               |
| Forfeited                             | (8 )             | \$ 34.85                               |
| Unvested balance at June 30, 2017     | 810              | \$ 31.20                               |

During the six months ended June 30, 2017, the Company issued 100,692 RSUs to employees. These RSUs are subject to time-based vesting. As of June 30, 2017, there was approximately \$5.4 million of unrecognized

compensation cost related to

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the time-based RSUs, which the Company expects to recognize over a remaining weighted-average period of 1.88 years. 238,781 restricted stock units subject to time-based vesting remained unvested and outstanding at June 30, 2017.

During the six months ended June 30, 2017, the Company issued 20,041 RSUs to employees. These RSUs are subject to performance-based vesting conditions, and vesting accelerates upon the occurrence of certain milestone events. In September 2019, any of these unvested RSUs will vest. As a result, when 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. At June 30, 2017, there was approximately \$6.7 million of unrecognized compensation cost related to the performance-based RSUs, which the Company expects to recognize over a remaining weighted-average period of 1.33 years. All 570,818 of these performance-based RSUs remained outstanding at June 30, 2017.

**Employee Stock Purchase Plan**

During the three months ended June 30, 2017 and 2016, the Company recorded \$0.1 million and \$0.1 million, respectively, and during the six months ended June 30, 2017 and 2016, the Company recorded \$0.1 million and \$0.1 million of stock-based compensation expense related to the 2013 Employee Stock Purchase Plan (ESPP).

**17. Income Taxes**

For the three and six months ended June 30, 2017, the Company recorded current income tax expense of \$6 thousand and \$12 thousand, respectively, related to state income taxes on its interest income. For the three and six months ended June 30, 2016, the Company did not record a current or deferred income tax provision or benefit.

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 June 30, 2017 and December 31, 2016.

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, 2013 through December 31, 2016. 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 June 30, 2017 and December 31, 2016, the Company did not have any significant uncertain tax positions.

**18. Related Party Transactions****Celgene Corporation**

In connection with prior arrangements, Celgene owned 12.6% and 12.8% of the Company's fully diluted equity as of June 30, 2017 and December 31, 2016, respectively. Refer to Note 15 for additional information regarding this collaboration arrangement.

During the three and six months ended June 30, 2017 and 2016, all revenue recognized by the Company was recognized under the Celgene collaboration arrangement and, as of June 30, 2017, the Company had \$4.0 million of deferred revenue related to the Celgene collaboration arrangement.

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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, 2016.

Certain matters discussed in this Quarterly Report on Form 10-Q may be deemed to be forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. In this Quarterly Report on Form 10-Q, words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "project," "should," "strategy," "target," "will," "would," or, in each case, the negative or other variations thereon or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

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

- our ongoing and planned preclinical studies and clinical trials;
- clinical trial data and the timing of results of our ongoing clinical trials;
- our plans to develop and commercialize ACE-083 and our 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 commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position; and
- our estimates regarding our operating capital requirements, 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, 2016 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.

You should read the following discussion and analysis of financial condition and results of operations together with Part I Item 1 "Financial Statements" and related notes included elsewhere in this Quarterly Report on Form 10-Q.

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## Overview

We are a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative 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 built a highly productive discovery and development platform that has generated innovative therapeutic candidates with novel mechanisms of action. These differentiated therapeutic candidates have the potential to significantly improve clinical outcomes for patients across many fields of medicine.

Luspatercept, our lead program, and sotatercept, are partnered with Celgene Corporation, or Celgene. Luspatercept is designed to promote red blood cell production through a novel mechanism, and we are developing luspatercept with Celgene to treat anemia and associated complications in myelodysplastic syndromes (MDS), beta-thalassemia, and myelofibrosis. Celgene is currently conducting two Phase 3 clinical trials for luspatercept: one for the treatment of patients with lower risk MDS, the "MEDALIST" trial, and another for the treatment of patients with beta-thalassemia, the "BELIEVE" trial. We are evaluating opportunities for the development of sotatercept. Celgene is responsible for paying 100% of the development costs for all clinical trials for luspatercept and sotatercept. We may receive up to an additional \$545.0 million of potential development, regulatory and commercial milestone payments and, if these therapeutic candidates are commercialized, we will receive a royalty on net sales in the low-to-mid 20% range. We will co-promote luspatercept and sotatercept, if approved, in North America for which our commercialization costs will be entirely funded by Celgene.

We wholly own and independently developing ACE-083. ACE-083 is designed for the treatment of focal muscle disorders, and we are currently conducting a Phase 2 clinical trial with ACE-083 in patients with facioscapulohumeral dystrophy. In August 2017, we announced that we have dosed the first patient in a Phase 2 clinical trial with ACE-083 in Charcot-Marie-Tooth disease. We previously reported data from the Phase 1 clinical trial of ACE-083 showing marked increases in the volume of muscles treated with ACE-083 measured using MRI.

In addition to our clinical programs, we are conducting research to identify new therapeutic candidates to bring forward into clinical trials. To this end, we implemented a new platform technology, IntelliTrap™, that is expanding our discovery efforts. We have nominated an IntelliTrap™ molecule, ACE-2494, as a candidate for clinical development. As of June 30, 2017, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$337.3 million from public investors, \$96.2 million in equity investments from our collaboration partners and \$268.0 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, 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;
- seek regulatory approval for our therapeutic candidates; and
- operate as a public company.

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 sotatercept and luspatercept are paid by Celgene. If we obtain regulatory approval for ACE-083, 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.

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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, 2016.

### Financial Operations Overview

#### Revenue

##### Collaboration Revenue

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

#### Costs and Expenses

##### Research and Development Expenses

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

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

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

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

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

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





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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 June 30, 2017, we have incurred \$508.4 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 sotatercept and luspatercept are reimbursed 100% by Celgene. These reimbursements are recorded as revenue. We are expensing the costs of eight Phase 2 clinical trials for luspatercept, dalantercept and ACE-083, of which the four for luspatercept are reimbursed by Celgene and the two for dalantercept 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 and six months ended June 30, 2017 and 2016 are as follows:

|  | Three Months |          | Six Months |          |
|--|--------------|----------|------------|----------|
|  | Ended        |          | Ended      |          |
| (in thousands)                                 | June 30,     |          | June 30,   |          |
|  | 2017         | 2016     | 2017       | 2016     |
| Luspatercept(1)                                | \$1,456      | \$1,703  | \$3,453    | \$3,319  |
| Dalantercept(2)                                | 1,454        | 2,133    | 2,750      | 4,087    |
| ACE-083  | 3,157        | 737      | 5,185      | 1,614    |
| ACE-2494                                       | 1,161        | —        | 2,447      | —        |
| Total direct research and development expenses | 7,228        | 4,573    | 13,835     | 9,020    |
| Other expenses(3)                              | 14,370       | 11,565   | 29,492     | 23,370   |
| Total research and development expenses        | \$21,598     | \$16,138 | \$43,327   | \$32,390 |

(1) Expenses associated with luspatercept are reimbursed 100% by Celgene.

(2) Development of dalantercept is being discontinued.

(3) 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 (Expense) Income, Net

Other (expense) income, 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-

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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 preferred stock or common stock underlying the warrants. The Black-Scholes option pricing model was used at June 30, 2017.

**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.

There have been no material changes to our critical accounting policies since December 31, 2016. 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, 2016.

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## Results of Operations

## Comparison of the Three Months Ended June 30, 2017 and 2016

| (in thousands)              | Three Months Ended |            | Increase<br>(Decrease) |
|-----------------------------|--------------------|------------|------------------------|
|                             | June 30,<br>2017   | 2016       |                        |
| Revenue:                    |                    |            |                        |
| Collaboration revenue:      |                    |            |                        |
| License and milestone       | \$ 135             | \$ 135     | \$ —                   |
| Cost-sharing, net           | 2,922              | 3,060      | (138 )                 |
| Total revenue               | 3,057              | 3,195      | (138 )                 |
| Costs and expenses:         |                    |            |                        |
| Research and development    | 21,598             | 16,138     | 5,460                  |
| General and administrative  | 11,370             | 6,712      | 4,658                  |
| Total costs and expenses    | 32,968             | 22,850     | 10,118                 |
| Loss from operations        | (29,911 )          | (19,655 )  | (10,256 )              |
| Other income (expense), net | 248                | (2,361 )   | 2,609                  |
| Loss before income taxes    | (29,663 )          | (22,016 )  | (7,647 )               |
| Income tax expense          | (6 )               | —          | (6 )                   |
| Net loss                    | \$(29,669)         | \$(22,016) | \$(7,653 )             |

Revenue. We recognized revenue of \$3.1 million in the three months ended June 30, 2017, compared to \$3.2 million in the same period in 2016. All of the revenue in both periods was derived from the Celgene agreements. This \$0.1 million decrease is attributable to miscellaneous reductions in external expenses partially offset by an increase in reimbursement for personnel compared to the prior year.

Research and Development Expenses. Research and development expenses were \$21.6 million in the three months ended June 30, 2017, compared to \$16.1 million in the same period in 2016. This \$5.5 million increase was primarily due to an increase in personnel expenses totaling \$3.0 million, to support development of our wholly owned therapeutic candidates and preclinical programs, which includes an increase in stock-based compensation expense of \$1.6 million. Other increases include clinical trial and toxicology expenses of \$3.0 million. These increases were partially offset by a decrease in miscellaneous research and drug supply expense of \$0.6 million.

General and Administrative Expenses. General and administrative expenses were \$11.4 million in the three months ended June 30, 2017, compared to \$6.7 million for the same period in 2016. This \$4.7 million increase was primarily due to an increase in personnel expense of \$4.8 million, which includes an increase in stock-based compensation primarily due to modifications of a former executive officer's equity awards, which was announced in May 2017.

Other Income (Expense), Net. Other income, net was \$0.2 million in the three months ended June 30, 2017, compared to other expense, net of \$2.4 million for the same period in 2016. This \$2.6 million change was primarily due to a \$2.6 million decrease in the loss associated with marking the common warrant liability to market in each period.

Income Tax Expense. Income tax expense is attributable to taxes on interest income from our investment portfolio.

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## Comparison of the Six Months Ended June 30, 2017 and 2016

| (in thousands)             | Six Months Ended |            | Increase    |
|----------------------------|------------------|------------|-------------|
|                            | June 30,<br>2017 | 2016       | (Decrease)  |
| Revenue:                   |                  |            |             |
| Collaboration revenue:     |                  |            |             |
| License and milestone      | \$271            | \$15,279   | \$(15,008 ) |
| Cost-sharing, net          | 6,491            | 6,117      | 374         |
| Total revenue              | 6,762            | 21,396     | (14,634 )   |
| Costs and expenses:        |                  |            |             |
| Research and development   | 43,327           | 32,390     | 10,937      |
| General and administrative | 19,203           | 12,618     | 6,585       |
| Total costs and expenses   | 62,530           | 45,008     | 17,522      |
| Loss from operations       | (55,768 )        | (23,612 )  | (32,156 )   |
| Other income, net          | 705              | 6,656      | (5,951 )    |
| Loss before income taxes   | (55,063 )        | (16,956 )  | (38,107 )   |
| Income tax expense         | (12 )            | —          | (12 )       |
| Net loss                   | \$(55,075)       | \$(16,956) | \$(38,119)  |

Revenue. We recognized revenue of \$6.8 million in the six months ended June 30, 2017, compared to \$21.4 million in the same period in 2016. All of the revenue in both periods was derived from the Celgene agreements. This \$14.6 million decrease was primarily due to the receipt of a \$15 million milestone payment from Celgene for the initiation of a Phase 3 clinical trial with luspatercept in 2016, offset in part by an increase in cost sharing revenue of \$0.4 million primarily related to an increase in reimbursement for personnel compared to the prior year.

Research and Development Expenses. Research and development expenses were \$43.3 million in the six months ended June 30, 2017, compared to \$32.4 million in the same period in 2016. This \$10.9 million increase was primarily due to an increase in personnel expenses totaling \$6.4 million, to support development of our wholly owned therapeutic candidates and preclinical programs, which includes an increase in stock-based compensation expense of \$3.4 million. Other increases include miscellaneous research and drug supply expenses of \$0.8 million and clinical trial and toxicology expenses of \$4.6 million. These increases were partially offset by a decrease in licensing expense related to payments that we made in connection with the achievement of a milestone in 2016 totaling \$0.9 million.

General and Administrative Expenses. General and administrative expenses were \$19.2 million in the six months ended June 30, 2017, compared to \$12.6 million for the same period in 2016. This \$6.6 million increase was primarily due to an increase in personnel expense of \$6.9 million, which includes an increase in stock-based compensation expense of \$4.0 million, primarily due to modifications of a former executive officer's equity awards, which was announced in May 2017.

Other Income, Net. Other income, net was \$0.7 million in the six months ended June 30, 2017, compared to \$6.7 million for the same period in 2016. This \$6.0 million decrease was primarily due to a \$6.1 million decrease in the gain associated with marking the common warrant liability to market.

Income Tax Expense. Income tax provision is attributable to taxes on interest 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 June 30, 2017, we had an accumulated deficit of \$419.6 million. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of the sale of equity, debt financings or other sources, including potential additional collaborations.

As of June 30, 2017, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$337.3 million from public investors, \$96.2 million in equity investments from our collaboration partners

and \$268.0 million in upfront payments, milestones, and net research and development payments from our collaboration partners.

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As of June 30, 2017, we had \$194.0 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 the second half of 2019.

**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)                            | Six Months Ended |            |
|---|------------------|------------|
|   | June 30,<br>2017 | 2016       |
| Net cash (used in) provided by:           |                  |            |
| Operating activities                      | \$(39,110)       | \$(14,933) |
| Investing activities                      | 47,118           | (118,180)  |
| Financing activities                      | 1,768            | 142,325    |
| Net increase in cash and cash equivalents | \$9,776          | \$9,212    |

**Operating Activities**

Net cash used in operating activities was \$39.1 million for the six months ended June 30, 2017 compared to \$14.9 million during the same period in 2016. The change was driven primarily by an increase in net loss of \$38.1 million, which includes lower revenue due to a \$15.0 million milestone recognized in 2016 and a \$6.1 million decrease in the gain associated with marking the common warrants to market. Operating expenses increased by \$17.5 million for 2017, including a \$7.4 million increase in non-cash stock-based compensation expense, in part due to modifications of the equity awards of our Chief Operating Officer.

**Investing Activities**

Net cash provided by investing activities was \$47.1 million for the six months ended June 30, 2017 compared to net cash used in investing activities of \$118.2 million for the six months ended June 30, 2016. This increase in net cash provided by investing activities was primarily due to net maturities of our investments of \$50.0 million during the six months ended June 30, 2017 in connection with managing the portfolio to meet our cash requirements, compared to net purchases of \$116.6 million in the six months ended June 30, 2016, in connection with implementing our investment policy.

**Financing Activities**

Net cash provided by financing activities was \$1.8 million for the six months ended June 30, 2017 compared to \$142.3 million for the same period in 2016. The decrease is primarily attributable to \$140.3 million of proceeds received during the comparable period in 2016 from our public offering.

**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, 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 the second half of 2019. 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

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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 \$136.5 million as of December 31, 2016, 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, 2016, we had federal NOL carryforwards of approximately \$339.3 million and state NOL carryforwards of \$293.7 million available to reduce future taxable income, if any. These federal and state NOL carryforwards expire at various times through 2036. 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, 2016.

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### Contractual Obligations and Commitments

During the three months ended June 30, 2017, 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, 2016 except as described below.

On March 16, 2017, the Company entered into an approximately six and a half year lease for approximately 11,825 square feet of rentable office and lab space located at 99 Erie Street, Cambridge, Massachusetts. The lease commenced on May 1, 2017 and ends on September 30, 2023. Excluding operating costs and real estate taxes, rent for the first year is \$0.7 million with annual rent escalations thereafter. The total operating lease obligation for the term of this agreement is \$5.1 million, excluding operating costs and real estate taxes. In addition, the lease provides an optional contribution from the landlord towards build-out of the space of up to \$0.1 million. The Company has the option to extend the lease by an additional three years. In accordance with the lease, the Company entered into a cash-collateralized, irrevocable standby letter of credit in the amount of \$0.2 million, naming the landlord as beneficiary.

### 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 June 30, 2017 and December 31, 2016, we had cash, cash equivalents and investments of \$194.0 million and \$234.4 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 June 30, 2017, 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

June 30, 2017, the design and operation of our disclosure controls and procedures were effective.

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Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rule 13a-15(f) under the Exchange Act) during the quarter ended June 30, 2017, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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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, 2016.

Item 6. Exhibits

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

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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: August 3, 2017      By:      /s/ HABIB J. DABLE  
Habib J. Dable  
Chief Executive Officer, President and Director

Date: August 3, 2017      By:      /s/ KEVIN F. MCLAUGHLIN  
Kevin F. McLaughlin  
Senior Vice President, Chief Financial Officer and  
Treasurer



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EXHIBIT INDEX

| Exhibit<br>Number | Description of Exhibit   |
|-------------------|--|
| 10.1*             | Amendment to Amended and Restated Employment Agreement between Steven D. Ertel and Acceleron Pharma Inc., dated as of April 28, 2017 (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36065), filed on May 2, 2017) |
| 31.1              | Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002   |
| 31.2              | Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002   |
| 32.1              | Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002  |
| 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   |

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\*Management contract or compensatory plan or arrangement.