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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 March 31, 2020**

**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 incorporation or organization) | (Primary Standard Industrial Classification Code Number) | (I.R.S. Employer Identification Number) |

**128 Sidney Street**
**Cambridge, MA 02139**
**(617) 649-9200**
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

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

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

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** x **No** o

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

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 | x | Accelerated filer | o |
| Non-accelerated filer | o | 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. o

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

As of April 30, 2020, there were 53,824,625 shares of the registrant's Common Stock, par value $0.001 per share, outstanding.

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**PART I. FINANCIAL INFORMATION**

**Item 1. Financial Statements**

**Acceleron Pharma Inc.**

**Condensed Consolidated Balance Sheets**

**(amounts in thousands, except share and per share data)**

**(unaudited)**

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 277,486 | $ 237,677 |
| Collaboration receivables (all amounts are with a related party) | 8,435 | 8,547 |
| Prepaid expenses and other current assets | 9,284 | 10,000 |
| Short-term investments | 136,954 | 193,692 |
| Total current assets | 432,159 | 449,916 |
| Property and equipment, net | 6,668 | 6,812 |
| Operating lease - right of use asset, net | 22,531 | 23,908 |
| Other assets | 1,681 | 1,793 |
| Long-term investments | 1,123 | 22,477 |
| Total assets | $ 464,162 | $ 504,906 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 3,929 | $ 2,295 |
| Accrued expenses | 18,189 | 24,895 |
| Operating lease liability - right of use | 6,412 | 6,183 |
| Total current liabilities | 28,530 | 33,373 |
| Operating lease liability - right of use, net of current portion | 18,506 | 20,201 |
| Warrants to purchase common stock | 3,304 | 1,856 |
| Total liabilities | 50,340 | 55,430 |
| Commitments and contingencies (Note 13) | | |
| Stockholders' equity: | | |
| Undesignated preferred stock, $0.001 par value: 25,000,000 shares authorized and no shares issued or outstanding | — | — |
| Common stock, $0.001 par value: 175,000,000 shares authorized; 53,519,920 and 53,123,567 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively | 53 | 53 |
| Additional paid-in capital | 1,176,359 | 1,160,807 |
| Accumulated deficit | (762,346) | (711,407) |
| Accumulated other comprehensive (loss) income | (244) | 23 |
| Total stockholders' equity | 413,822 | 449,476 |
| Total liabilities and stockholders' equity | $ 464,162 | $ 504,906 |

See accompanying notes to these condensed consolidated financial statements.

**Acceleron Pharma Inc.**

**Condensed Consolidated Statements of Operations and Comprehensive Loss**

**(amounts in thousands, except per share data)**

**(unaudited)**

| | Three Months Ended March 31, | |
|---|---|---|
| | **2020** | **2019** |
| Revenue: | | |
| Collaboration revenue: | | |
| Cost-sharing, net | 2,824 | 2,780 |
| Royalty | 1,520 | — |
| Total revenue (all amounts are with a related party) | 4,344 | 2,780 |
| Costs and expenses: | | |
| Research and development | 37,663 | 32,771 |
| Selling, general and administrative | 18,253 | 10,814 |
| Total costs and expenses | 55,916 | 43,585 |
| Loss from operations | (51,572) | (40,805) |
| Other income, net | 648 | 2,772 |
| Loss before income taxes | (50,924) | (38,033) |
| Income tax provision | (15) | (20) |
| Net loss | $ (50,939) | $ (38,053) |
| Other comprehensive (loss) income: | | |
| Net unrealized holding (losses) gains on short-term and long-term investments during the period, net of tax | (267) | 268 |
| Comprehensive loss | $ (51,206) | $ (37,785) |
| | | |
| Net loss per share- basic and diluted | $ (0.95) | $ (0.74) |
| | | |
| Weighted-average number of common shares used in computing net loss per share- basic and diluted | 53,361 | 51,126 |

See accompanying notes to these condensed consolidated financial statements.

**Acceleron Pharma Inc.**

**Condensed Consolidated Statements of Stockholders' Equity**

**(amounts in thousands, except share and per share data)**

**(unaudited)**

**Three Months Ended March 31, 2020**

| | Common Stock | | | | | |
|---|---|---|---|---|---|---|
| | Number of Shares | $0.001 Par Value | Additional Paid-In Capital | Accumulated Deficit | Comprehensive Income (Loss) | Total Stockholders' Equity |
| Balance at December 31, 2019 | 53,123,567 | $ 53 | $ 1,160,807 | $ (711,407) | $ 23 | $ 449,476 |
| Stock-based compensation | — | — | 6,679 | — | — | 6,679 |
| Exercise of stock options | 295,757 | — | 8,485 | — | — | 8,485 |
| Vesting of restricted stock units, net of shares withheld for taxes | 77,949 | — | (472) | — | — | (472) |
| Issuance of common stock related to ESPP | 22,647 | — | 860 | — | — | 860 |
| Unrealized loss on available-for-sale securities, net of tax | — | — | — | — | (267) | (267) |
| Net loss | — | — | — | (50,939) | — | (50,939) |
| Balance at March 31, 2020 | 53,519,920 | $ 53 | $ 1,176,359 | $ (762,346) | (244) | $ 413,822 |

**Three Months Ended March 31, 2019**

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

**Acceleron Pharma Inc.**

**Condensed Consolidated Statements of Cash Flows**

**(amounts in thousands)**

**(unaudited)**

| | Three Months Ended March 31, | |
|---|---|---|
| | **2020** | **2019** |
| **Operating Activities** | | |
| Net loss | $ (50,939) | $ (38,053) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Depreciation and amortization | 996 | 933 |
| Stock-based compensation | 6,679 | 6,992 |
| Other non-cash items | 1,365 | 959 |
| Changes in assets and liabilities: | | |
| Prepaid expenses and other assets | 828 | (237) |
| Collaboration receivables (all amounts are with a related party) | 112 | 4,259 |
| Non-cash lease expense(1) | 1,377 | 1,234 |
| Accounts payable | 1,634 | 3,466 |
| Accrued expenses | (7,148) | (4,544) |
| Operating lease obligations | (1,466) | (1,317) |
| Other changes in operating assets and liabilities | 17 | — |
| Net cash used in operating activities | (46,545) | (26,308) |
| **Investing Activities** | | |
| Purchases of investments | (10,454) | (216,116) |
| Proceeds from sales and maturities of investments | 88,361 | 64,133 |
| Purchases of property and equipment | (426) | (587) |
| Net cash provided by (used in) investing activities | 77,481 | (152,570) |
| **Financing Activities** | | |
| Proceeds from issuance of common stock from public offering, net of issuance costs | — | 248,155 |
| Net proceeds from exercises and vesting of stock awards, ESPP contributions, and exercise of warrants to purchase common stock | 8,873 | 1,161 |
| Net cash provided by financing activities | 8,873 | 249,316 |
| Net increase in cash, cash equivalents and restricted cash | 39,809 | 70,438 |
| Cash, cash equivalents and restricted cash at beginning of period | 239,274 | 145,649 |
| Cash, cash equivalents and restricted cash at end of period | $ 279,083 | $ 216,087 |
| **Supplemental Disclosure of Non-Cash Investing and Financing Activities:** | | |
| Purchase of property and equipment included in accounts payable and accrued expenses | $ 425 | $ 267 |

(1) The Company has reclassified prior period amounts to conform with presentation as of March 31, 2020.

See accompanying notes to these condensed consolidated financial statements.

**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 biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases. The Company's leadership in the understanding of TGF-beta biology and protein engineering generates innovative compounds that engage the body's ability to regulate cellular growth and repair.

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

## 2. Basis of Presentation

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

The accompanying interim condensed consolidated financial statements are unaudited and reflect the application of certain significant accounting policies as described below and elsewhere in these notes to the financial statements. As of March 31, 2020, 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, 2019, have not changed, and the unaudited interim financial statements have been prepared on the same basis as the audited annual financial statements as of and for the year ended December 31, 2019. In the opinion of management, the accompanying interim condensed consolidated financial statements reflect all adjustments, consisting of normal recurring adjustments, necessary for the fair presentation of the Company's financial position as of March 31, 2020, the results of its operations for the three months ended March 31, 2020 and 2019, and its cash flows for the three months ended March 31, 2020 and 2019.

The accompanying interim condensed consolidated financial statements include the results of operations of the Company and its wholly-owned subsidiaries. All intercompany accounts and transactions have been eliminated in consolidation.

The results for the three months ended March 31, 2020 are not necessarily indicative of the results to be expected for the year ending December 31, 2020, 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, 2019, 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, 2019.

## 3. Use of Estimates

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

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

**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 value.

The Company determines the appropriate classification of marketable securities at the time of purchase and reevaluates such designation at each balance sheet date. The Company has classified all of its marketable securities at March 31, 2020 as "available-for-sale" pursuant to ASC 320, *Investments – Debt and Equity Securities.* 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.

In June 2016, the FASB issued Accounting Standards Update 2016-13, *Financial Instruments-Credit Losses*. The new standard requires an estimate of expected credit losses only when the fair value of an available-for-sale debt security is below its amortized cost basis, and credit losses are limited to the amount by which the security's amortized cost basis exceeds its fair value. Credit-related impairment is recognized as an allowance for credit losses on the balance sheet with a corresponding adjustment to earnings.

The standard additionally requires an investor to determine whether a decline in the fair value below the amortized cost basis of an available-for-sale debt security is due to credit-related factors or noncredit-related factors. Any impairment that is not credit related is recognized in other comprehensive income, net of applicable taxes. The Company adopted ASU 2016-13 effective January 1, 2020, with no material impact on its consolidated financial statements and related disclosures.

The following is a summary of available-for-sale securities with unrealized losses as of March 31, 2020 (in thousands):

| | Less than 12 months | |
|---|---|---|
| | Fair Value | Unrealized Losses |
| Corporate obligations | $ 50,840 | $ (181) |
| Total available-for sale securities in an unrealized loss position | $ 50,840 | $ (181) |

There were no securities in an unrealized loss for greater than 12 months as of March 31, 2020. The unrealized losses on the Company's corporate obligations were caused by central bank and market interest rate decreases on securities purchased at a premium. The contractual terms of these investments do not permit the issuer to settle the securities at a price less than the amortized cost bases of the investments. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost bases. The Company did not record an allowance for credit losses as of March 31, 2020.

Prior to January 1, 2020, the Company reviewed marketable securities for other-than-temporary impairment whenever the fair value of a marketable security was less than the amortized cost and evidence indicated that a marketable security's carrying amount was not recoverable within a reasonable period of time. Other-than-temporary impairments of investments were recognized in the consolidated statements of operations if the Company had experienced a credit loss, had the intent to sell the marketable security, or if it was more likely than not that the Company would be required to sell the marketable security before recovery of the amortized cost basis. Evidence considered in this assessment included 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 December 31, 2019 was $35.8 million. The aggregate fair value of securities held by the Company in an unrealized loss position for more than twelve months as of December 31, 2019 was zero. The aggregate unrealized loss for those securities in an unrealized loss position for more than twelve months was zero. The Company determined it did not hold any investments with any other-than-temporary impairment as of December 31, 2019.

The following is a summary of cash, cash equivalents and available-for-sale securities as of March 31, 2020 and December 31, 2019 (in thousands):

| | March 31, 2020 | | | |
|---|---|---|---|---|
| | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Estimated Fair Value |
| Cash and cash equivalents due in 90 days or less | $ 277,485 | $ 1 | $ — | $ 277,486 |
| Available-for-sale securities: | | | | |
| Corporate obligations | 97,260 | 56 | (181) | 97,135 |
| U.S. Treasury securities | 32,545 | 147 | — | 32,692 |
| Certificates of deposit | 245 | 3 | — | 248 |
| Mortgage and other asset backed securities | 8,000 | 2 | — | 8,002 |
| Total available-for-sale securities | $ 138,050 | $ 208 | $ (181) | $ 138,077 |
| Total cash, cash equivalents and available-for-sale securities | $ 415,535 | $ 209 | $ (181) | $ 415,563 |

| | December 31, 2019 | | | |
|---|---|---|---|---|
| | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Estimated Fair Value |
| Cash and cash equivalents due in 90 days or less | $ 237,677 | $ — | $ — | $ 237,677 |
| Available-for-sale securities: | | | | |
| Corporate obligations | 124,676 | 219 | (15) | 124,880 |
| U.S. Treasury securities | 78,230 | 98 | (1) | 78,327 |
| Certificates of deposit | 490 | 3 | — | 493 |
| Mortgage and other asset backed securities | 12,476 | 5 | (12) | 12,469 |
| Total available-for-sale securities | $ 215,872 | $ 325 | $ (28) | $ 216,169 |
| Total cash, cash equivalents and available-for-sale securities | $ 453,549 | $ 325 | $ (28) | $ 453,846 |

## 6. Restricted Cash

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

| | March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Cash and cash equivalents | $ 277,486 | $ 214,490 |
| Restricted cash | 1,597 | 1,597 |
| Total cash, cash equivalents and restricted cash shown in the statement of cash flows | $ 279,083 | $ 216,087 |

As of March 31, 2020 and December 31, 2019, the Company maintained letters of credit totaling $1.6 million held in the form of certificates of deposit and money market funds as collateral for the Company's facility lease obligation and its credit cards.

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

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

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

The Company routinely assesses the creditworthiness of its collaboration partner. 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 allowance for credit losses has been recorded for the Company's collaboration receivables as of March 31, 2020.

**8. Fair Value Measurements**

The following tables set forth the Company's financial instruments carried at fair value using the lowest level of input that is significant to each financial instrument as of March 31, 2020 and December 31, 2019 (in thousands):

| | March 31, 2020 | | | |
|---|---|---|---|---|
| | Quoted Prices in Active Markets for Identical Items (Level 1) | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) | Total |
| Assets: | | | | |
| Money market funds | $ 239,753 | $ — | $ — | $ 239,753 |
| Corporate obligations | — | 101,130 | — | 101,130 |
| U.S. Treasury securities | — | 36,691 | — | 36,691 |
| Certificates of deposit | — | 248 | — | 248 |
| Mortgage and other asset backed securities | — | 8,002 | — | 8,002 |
| Total assets | $ 239,753 | $ 146,071 | $ — | $ 385,824 |
| Liabilities: | | | | |
| Warrants to purchase common stock | $ — | $ — | $ 3,304 | $ 3,304 |
| Total liabilities | $ — | $ — | $ 3,304 | $ 3,304 |

| | December 31, 2019 | | | |
|---|---|---|---|---|
| | Quoted Prices in Active Markets for Identical Items (Level 1) | Significant other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) | Total |
| Assets: | | | | |
| Money market funds | $ 193,867 | $ — | $ — | $ 193,867 |
| Corporate obligations | — | 138,369 | — | 138,369 |
| U.S. Treasury securities | — | 83,819 | — | 83,819 |
| Certificates of deposit | — | 493 | — | 493 |
| Mortgage and other asset backed securities | — | 12,470 | — | 12,470 |
| Total assets | $ 193,867 | $ 235,151 | $ — | $ 429,018 |
| Liabilities: | | | | |
| Warrants to purchase common stock | $ — | $ — | $ 1,856 | $ 1,856 |
| Total liabilities | $ — | $ — | $ 1,856 | $ 1,856 |

The money market funds noted above are included in cash and cash equivalents in the accompanying condensed consolidated balance sheets. The Company recognizes transfers between levels of the fair value hierarchy as of the end of the reporting period. There were no transfers within the hierarchy during the three months ended March 31, 2020 or the year ended December 31, 2019.

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

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

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Beginning balance | $ 1,856 | $ 1,491 |
| Change in fair value | 1,448 | 116 |
| Ending balance | $ 3,304 | $ 1,607 |

The fair value of the warrants to purchase common stock on the date of issuance and on each re-measurement date for those warrants classified as liabilities was estimated using either the Monte Carlo simulation framework, which incorporates future financing events over the remaining life of the warrants to purchase common stock, or for certain re-measurement dates, due to the warrants being deeply in the money, the Black-Scholes option pricing model. Due to the nature of these inputs, the valuation of the warrants is considered a Level 3 measurement. At each reporting period, the Company evaluates the best valuation methodology. At March 31, 2020, the Black-Scholes option pricing model was used.

**9. Net Loss Per Share**

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

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Outstanding stock options | 4,232 | 4,115 |
| Common stock warrants | 39 | 39 |
| Shares issuable under employee stock purchase plan | 13 | 13 |
| Outstanding restricted stock units (1) | 557 | 630 |
| | 4,841 | 4,797 |

(1) This balance is comprised of both the restricted stock units and performance-based restricted stock units described in Note 15.

**10. Comprehensive Loss**

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions, other events, and circumstances from non-owner sources. Comprehensive loss consists of net loss and other comprehensive (loss) income, 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 and losses on investments as of March 31, 2020 and December 31, 2019.

**11. Recent Accounting Pronouncements**

*Recently Adopted*

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments-Credit Losses*. The new standard requires entities to measure all expected credit losses for financial assets held at the reporting date based on historical experience, current conditions and reasonable and supportable forecasts. For available-for-sale debt securities with unrealized losses, the standard requires allowances to be recorded instead of reducing the amortized cost of the investment. The standard limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which the carrying value exceeds fair value and requires the reversal of previously recognized credit losses if fair value increases. On January 1, 2020 the Company adopted ASU 2016-13. For discussion regarding the impact of this accounting pronouncement and its amendments, refer to Note 5 within the notes to our consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

In June 2018, the FASB issued ASU 2018-15, *Intangible-Goodwill and Other Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement that is a Service Contract.* This amendment aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use

software. On January 1, 2020, the Company adopted ASU 2018-15 on a prospective basis, with no material impact on its consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU 2019-12, *Simplifying the Accounting for Income Taxes*. The ASU simplifies the accounting for income taxes by eliminating certain exceptions to the guidance in ASC 740, *Income Taxes*, related to the approach for allocating income tax expense or benefit for the year to continuing operations, discontinued operations, other comprehensive income, and other charges or credits recorded directly to shareholders' equity; the methodology for calculating income taxes in an interim period; and the recognition of deferred tax liabilities for outside basis differences. On January 1, 2020, the Company early adopted ASU 2019-12 on a prospective basis, with no material impact on its consolidated financial statements and related disclosures.

## 12. Warrants

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

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

## 13. Commitments and Contingencies

### Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of its business. The Company was not subject to any material legal proceedings during the three months ended March 31, 2020, and, to the best of its knowledge, no material legal proceedings are currently pending or threatened.

### Other

The Company is also party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at March 31, 2020 and December 31, 2019, or royalties on future sales of specified products. See Note 14 for discussion of these arrangements.

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

## 14. Significant Agreements

### BMS (Bristol Myers Squibb Company)

***Overview***

On February 20, 2008, the Company entered into an agreement with Celgene, which was acquired by BMS in November 2019 and is now referred to herein as BMS, relating to sotatercept (the Original Sotatercept Agreement), which was amended on August 2, 2011 (as amended, the Amended Sotatercept Agreement). The Company further amended and restated the Original Sotatercept Agreement in its entirety on September 18, 2017 (the Restated Sotatercept Agreement) and clarified certain responsibilities of the Company and BMS in a letter agreement to the Restated Sotatercept Agreement on March 10, 2020. On August 2, 2011, the Company entered into a second agreement with BMS for REBLOZYL® (luspatercept-aamt) (the REBLOZYL Agreement, formerly the Luspatercept Agreement).

Since December 31, 2019, there have been no material changes to the key terms of the above agreements. For further information on the terms of the agreements, please see the notes to the consolidated financial statements included in the Company's Form 10-K for the year ended December 31, 2019.

***Accounting Analysis***

Upon adoption of ASC 606, all of the Company's performance obligations pursuant to its arrangements with BMS have been completed and all remaining potential milestone payments have been fully constrained as they relate to future regulatory events that are outside of the Company's control, and therefore the risk of significant reversal had not been resolved. As of March 31, 2020, the next milestone payment for REBLOZYL would be $25.0 million and would result from European Medicines Agency (EMA) approval of a Biologics License Application (BLA) or equivalent for luspatercept-aamt in either myelodysplastic syndromes or beta-thalassemia. The Company expects the EMA to issue a decision on the MAA in the second half of 2020. In accordance with the Company's accounting policy regarding revenue recognition as described in Note 2 to its Annual Report on Form 10-K, the revenue associated with this milestone will be recognized once it is probable that the application is approved by the regulatory authority. Milestone payments that are not within the control of the Company or the licensee are not considered probable of being achieved until those approvals are received. The approval of the application is not within the control of the Company or the licensee, and therefore, as of March 31, 2020, the Company cannot determine if it is probable that a regulatory agency will approve the applications.

Through March 31, 2020, under all BMS arrangements the Company has received net cost-share payments and milestones of $186.2 million and $44.8 million for REBLOZYL and sotatercept, respectively. The Company recorded net revenue of $4.3 million and $2.8 million during the three months ended March 31, 2020 and 2019, respectively.

**Other Agreements**

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. The Company also agreed to pay specified development milestone payments totaling up to $2.0 million for sotatercept and $0.7 million for REBLOZYL. In addition, the Company is obligated to pay milestone fees based on the Company's research and development progress, and U.S. sublicensing revenue ranging from 10%-25%, as well as royalties ranging from 1.0%-3.5% of net sales on any products under the licenses. During the three months ended March 31, 2020 and 2019, the Company expensed milestones, fees and royalties totaling $0.3 million and zero, respectively. Milestones and fees associated with development related activities are recorded as research and development expense. Costs related to royalties on sales of commercial products are recorded as selling, general and administrative expense.

In May 2014, the Company executed a collaboration agreement with a research technology company, and such collaboration agreement was amended and restated in March 2019. The Company paid an upfront research fee of $0.3 million upon execution of the original agreement. The Company also received an option to obtain a commercial license to the molecules developed during the collaboration. During the three months ended March 31, 2020 and 2019, the Company expensed milestones and fees totaling zero and $0.2 million, respectively, which is recorded as research and development expense.

In December 2019, the Company executed a license and collaboration agreement with Fulcrum Therapeutics to identify small molecules designed to modulate specific pathways associated with a targeted indication within the pulmonary disease space. The Company paid an upfront research fee of $10.0 million upon execution of this agreement. The Company also agreed to pay specified research, development and commercial milestone payments of up to $295.0 million for a first product commercialized and up to a maximum of $143.5 million in additional milestone payments for all subsequent products commercialized. Fulcrum will additionally receive tiered royalty payments in the mid-single-digit to low double-digit range on net sales, as well as reimbursement for relevant research and development costs. During the three months ended March 31, 2020 and 2019, the Company expensed milestones and fees totaling $0.3 million and zero, respectively, which is recorded as research and development expense.

**15. Stock-Based Compensation**

The Company recognized stock-based compensation expense related to the 2003 Stock Option and Restricted Stock Plan (the 2003 Plan), the 2013 Equity Incentive Plan (the 2013 Plan), and the 2013 Employee Stock Purchase Plan (the 2013 ESPP) in the consolidated statements of operations and comprehensive loss during the three months ended March 31, 2020 and 2019, respectively, as follows (in thousands):

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Research and development | $ 3,141 | $ 3,500 |
| Selling, general and administrative | 3,538 | 3,492 |
| | $ 6,679 | $ 6,992 |

**Stock Options**

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

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Expected volatility | 55.1% | 59.1% |
| Expected term (in years) | 6.0 | 6.0 |
| Risk-free interest rate | 1.6% | 2.6% |
| Expected dividend yield | —% | —% |

The following table summarizes the stock option activity under the Company's stock option plans during the three months ended March 31, 2020 (in thousands, except per share amounts and years):

| | Number of Stock Options | Weighted-Average Exercise Price Per Share | Weighted-Average Contractual Life (in years) | Aggregate Intrinsic Value(1) |
|---|---|---|---|---|
| Outstanding at December 31, 2019 | 3,820 | $ 36.26 | 6.81 | |
| Granted | 835 | $ 54.55 | | |
| Exercised | (296) | $ 28.69 | | |
| Canceled or forfeited | (127) | $ 41.03 | | |
| Outstanding at March 31, 2020 | 4,232 | $ 40.25 | 7.07 | $ 210,031 |
| Exercisable at March 31, 2020 | 2,328 | $ 34.86 | 5.48 | $ 128,039 |

(1) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in the money at March 31, 2020.

The aggregate intrinsic value of options exercised during the three months ended March 31, 2020 was $15.0 million.

As of March 31, 2020, there was $44.6 million of unrecognized compensation expense related to unvested stock options that is expected to be recognized over a weighted-average period of 2.84 years.

**Restricted Stock Units**

The following table summarizes the restricted stock unit (RSU) activity under the 2013 Plan during the three months ended March 31, 2020 (in thousands, except per share amounts):

| | Number of Stock Units | Weighted-Average Grant Date Fair Value Per Share |
|---|---|---|
| Unvested balance at December 31, 2019 | 397 | $ 39.20 |
| Granted | 197 | $ 54.31 |
| Vested | (86) | $ 38.69 |
| Forfeited | (26) | $ 41.36 |
| Unvested balance at March 31, 2020 | 482 | $ 45.36 |

As of March 31, 2020, there was approximately $18.2 million of related unrecognized compensation cost, which the Company expects to recognize over a remaining weighted-average period of 2.05 years.

**Performance-Based Restricted Stock Units**

On January 22, 2020, the Company granted performance-based restricted stock units (PSU) whereby vesting depends upon the occurrence of certain milestone events before December 31, 2022. As of March 31, 2020, none of the PSU milestones had been achieved. When achievement of a milestone becomes probable, compensation cost will be recognized from the grant date over the requisite service period. As of March 31, 2020, no related compensation cost had been recognized. The following table summarizes PSU activity under the 2013 Plan during the three months ended March 31, 2020 (in thousands, except per share amounts):

| | Number of Stock Units | Weighted-Average Grant Date Fair Value Per Share |
|---|---|---|
| Unvested balance at December 31, 2019 | — | $ — |
| Granted (1) | 78 | $ 52.99 |
| Vested | — | $ — |
| Forfeited | (3) | $ 52.99 |
| Unvested balance at March 31, 2020 | 75 | $ 52.99 |

(1) Pursuant to the terms of the awards granted on January 22, 2020, the actual number of awards earned could range between 0% and 200% of the number of awards granted.

As of March 31, 2020, there was approximately $4.0 million of related unrecognized compensation cost, which the Company expects to recognize over a remaining weighted-average period of 2.75 years. Depending on the actual number of awards earned, the actual expense recognized could range between 0% and 200% of this number.

**16. Income Taxes**

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

**17. Related Party Transactions**

**BMS**

BMS owned 11.8% and 12.0% of the Company's fully diluted equity as of March 31, 2020 and December 31, 2019, respectively. Refer to Note 14 for additional information regarding this collaboration arrangement.

During the three months ended March 31, 2020 and 2019, all revenue recognized by the Company was recognized under the BMS collaboration arrangement.

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

*The following information should be read in conjunction with the unaudited condensed consolidated financial statements and the notes thereto included in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2019.*

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

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

- *the impact on our business of the COVID-19 pandemic and the government's efforts to contain it;*
- *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 sotatercept in pulmonary hypertension and our other potential therapeutic candidates;*
- *our and Bristol Myers Squibb Company's, or BMS's, plans to develop and commercialize REBLOZYL® (luspatercept-aamt) and sotatercept outside of pulmonary hypertension;*
- *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 BMS;*
- *the timing of, and our and BMS's ability to, obtain and maintain regulatory approvals for our therapeutic candidates;*
- *the rate and degree of market acceptance and clinical utility of any approved therapeutic candidate, particularly in specific patient populations;*
- *our ability to quickly and efficiently identify and develop therapeutic candidates;*
- *our manufacturing capabilities and strategy;*
- *our plans for commercialization and marketing;*
- *our intellectual property position; and*
- *our estimates regarding our results of operations, financial condition, liquidity, capital requirements, prospects, growth and strategies.*

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

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

*You should also read carefully the factors described in the section "Item 1A. Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2019 to better understand the risks and uncertainties inherent in our business and*

*underlying any forward-looking statements. You are advised, however, to consult any further disclosures we make on related subjects in our subsequent Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, press releases, and our website.*

**Overview**

We are a biopharmaceutical company dedicated to the discovery, development and commercialization of 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 generate innovative therapeutic candidates, all of which encompass novel potential first-in-class mechanisms of action. If successful, these candidates could have the potential to significantly improve clinical outcomes for patients across these areas of high, unmet need.

We focus and prioritize our commercialization, research and development activities within two key therapeutic areas: hematology and pulmonary.

*Hematology*

Our first commercial product, REBLOZYL® (luspatercept-aamt), is a first-in-class erythroid maturation agent designed to promote red blood cell, or RBC, production through a novel mechanism, and is partnered with BMS (which acquired Celgene Corporation, or Celgene, in 2019). In November 2019, the U.S. Food and Drug Administration, or FDA, approved REBLOZYL for the treatment of anemia in adult patients with beta-thalassemia who require regular RBC transfusions. In April 2020, the FDA also approved REBLOZYL for the treatment of anemia failing an erythropoiesis stimulating agent and requiring two or more RBC units per eight weeks in adult patients with very low- to intermediate-risk myelodysplastic syndromes, or MDS, with ring sideroblasts or with a myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis. BMS has also submitted a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, for REBLOZYL. We and BMS recently announced that the Committee for Medicinal Products for Human Use, or CHMP, of the EMA has issued a positive opinion, recommending the approval of REBLOZYL for the treatment of adult patients with transfusion-dependent anemia due to very low-, low- and intermediate-risk MDS with ring sideroblasts, who had an unsatisfactory response or are ineligible for erythropoietin-based therapy, and adult patients with transfusion-dependent anemia associated with beta thalassemia. The CHMP recommendation will now be reviewed by the European Commission, which has the authority to approve medicines for the European Union. We expect the European Commission to issue a decision on the MAA for REBLOZYL in the second half of 2020.

BMS is currently conducting a Phase 2 clinical trial with luspatercept-aamt in non-transfusion-dependent beta-thalassemia patients, referred to as the BEYOND trial, with topline results currently expected by the end of 2020 or early 2021, and a Phase 3 clinical trial, the COMMANDS trial, in first-line, lower-risk MDS patients, with topline results expected in 2022. In myelofibrosis, BMS is conducting a Phase 2 clinical trial in patients with myelofibrosis-associated anemia, and initial results from this trial were presented in December 2019 at the 61st American Society of Hematology Annual Meeting and Exposition showing that luspatercept-aamt improved anemia in patients receiving and not receiving RBC transfusions, with more profound effects in patients treated with ruxolitinib, a small molecule JAK inhibitor. Based on these data, we and BMS announced plans to initiate by the end of 2020 the Phase 3 INDEPENDENCE study in patients with myelofibrosis-associated anemia who are being treated with JAK inhibitor therapy and require RBC transfusions.

If approved in the United States and Europe, we believe that there is an annual peak sales opportunity for REBLOZYL in excess of $2 billion in lower-risk MDS and beta-thalassemia, and upon successful development and approval in the United States and Europe, an additional $1 billion in myelofibrosis and other future development opportunities. We and BMS are evaluating luspatercept-aamt for the treatment of anemia in potential new indications that could provide additional sales opportunities.

BMS is responsible for paying 100% of the development costs for all clinical trials for luspatercept-aamt. We may receive a maximum of $125.0 million for remaining potential regulatory and commercial milestone payments. We have a co-promotion right in North America and our commercialization costs provided in the commercialization plan and budget approved by the Joint Commercialization Committee, or JCC, are entirely funded by BMS. Activities that we elect to conduct outside of the approved development or commercialization budgets to support REBLOZYL are at our own expense. We are eligible to receive tiered royalty payments from BMS on net sales of REBLOZYL in the low-to-mid 20% range.

*Pulmonary*

We are actively developing our lead pulmonary program, sotatercept, for the treatment of patients with pulmonary arterial hypertension, or PAH. Sotatercept is generally partnered with BMS, but we retain the exclusive rights to fund, develop, and lead the global commercialization of sotatercept in pulmonary hypertension, which we refer to as the PH field, and that includes

PAH. PAH is a rare and chronic, rapidly progressing disorder characterized by the constriction of small pulmonary arteries, resulting in abnormally high blood pressure in the pulmonary arteries.

In January 2020, we announced that the PULSAR Phase 2 clinical trial of sotatercept for the treatment of patients with PAH met its primary and key secondary endpoints, as well as other secondary endpoints. The 18-month extension period of the PULSAR trial is ongoing. We are also currently enrolling an exploratory study called SPECTRA to provide us with greater understanding of sotatercept's potential impact on PAH, with preliminary results expected in 2020. We also recently announced that the FDA has granted Breakthrough Therapy designation to sotatercept for the treatment of patients with PAH, and that the EMA has granted Priority Medicines, or PRIME, designation to sotatercept for the treatment of patients with PAH.

If sotatercept is commercialized to treat PAH and we recognize such revenue, then we will owe BMS a royalty in the low 20% range on global net sales. In certain circumstances, BMS may recognize revenue related to the commercialization of sotatercept in PAH, and in this scenario we will be eligible to receive a royalty from BMS such that the economic position of the parties is equivalent to the scenario in which we recognize such revenue.

*Funding and Expense*

As of March 31, 2020, our operations have been primarily funded by $105.1 million in equity investments from venture investors, $773.8 million from public investors, $154.1 million in equity investments from our collaboration partners and $361.2 million in upfront payments, milestones, and net research and development payments from our collaboration partners.

We expect to continue to incur significant expenses and 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 sotatercept in the PH field or any future therapeutic candidates;
- continue our preclinical studies and potential clinical development efforts of our existing preclinical therapeutic candidates;
- continue research activities for the discovery of new therapeutic candidates;
- manufacture therapeutic candidates for our preclinical studies and clinical trials, and potentially for commercialization;
- establish and maintain a sales, marketing and distribution infrastructure to commercialize any products for which we have or may obtain regulatory approval;
- acquire or in-license other therapeutic candidates and patents;
- seek regulatory approval for our therapeutic candidates; and
- attract and retain skilled personnel.

If we obtain regulatory approval for sotatercept in the PH field, or any future therapeutic candidate, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such costs are not paid by future partners. We will seek to fund our operations through the sale of equity, debt financings or other sources, including potential additional collaborations. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such other arrangements as, and when, needed, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our therapeutic candidates.

To date, we have only generated limited revenue from royalties on the sale of our first and only commercial product, REBLOZYL, since receiving our first regulatory approval from the FDA in November 2019. 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, 2019.

## Financial Operations Overview

### Revenue

***Collaboration Revenue***

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. We have generated limited revenue from royalties on the sale of products. Cost-sharing revenue represents amounts reimbursed by our collaboration partners for expenses incurred by us for research and development activities and co-promotion activities under our collaboration agreements. Cost-sharing revenue is recognized in the period that the related activities are performed. We recognize revenue from royalties when the related sales occur.

**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 beyond the initial approvals of REBLOZYL. 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 FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of the clinical development of our therapeutic candidates, or if we experience significant delays in the enrollment in any clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

From inception through March 31, 2020, we have incurred $850.3 million in research and development expenses. We plan to increase our research and development expenses for the foreseeable future as we continue the development of our TGF-beta platform therapeutic candidates, the discovery and development of preclinical therapeutic candidates, and the development of our clinical programs. Research and development expenses associated with luspatercept-aamt, and, outside of the PH field, sotatercept, are generally reimbursed 100% by BMS. These reimbursements are recorded as revenue. We are expensing the costs of Phase 2 clinical trials for luspatercept-aamt, sotatercept, and ACE-083, of which the luspatercept-aamt trials are reimbursed by BMS. Our Phase 2 clinical trials for ACE-083 are being discontinued. With respect to the luspatercept-aamt

clinical trials directly conducted by BMS, we do not incur and are not reimbursed for expenses related to these development activities.

We manage certain activities such as clinical trial operations, manufacture of therapeutic candidates, and preclinical animal toxicology studies through third-party CROs. The only costs we track by each therapeutic candidate are external costs such as services provided to us by CROs, manufacturing of preclinical and clinical drug product, and other outsourced research and development expenses. We do not assign or allocate to individual development programs internal costs such as salaries and benefits, facilities costs, lab supplies and the costs of preclinical research and studies, except for luspatercept-aamt costs for the purposes of billing BMS. Our external research and development expenses during the three months ended March 31, 2020 and 2019 are as follows:

| | Three Months Ended March 31, | |
|---|---|---|
| **(in thousands)** | **2020** | **2019** |
| Luspatercept-aamt(1) | $ 419 | $ 1,339 |
| Sotatercept(2) | 8,980 | 4,091 |
| ACE-083(3) | 4,187 | 4,250 |
| ACE-2494(4) | 6 | 769 |
| Total direct research and development expenses | 13,592 | 10,449 |
| Other expenses(5) | 24,071 | 22,322 |
| Total research and development expenses | $ 37,663 | $ 32,771 |

(1) These expenses associated with luspatercept-aamt are reimbursed 100% by BMS.

(2) These expenses are associated with our development of sotatercept in PAH.

(3) Development of ACE-083 is being discontinued. We expect to incur all remaining material expense by the end of 2020.

(4) Development of ACE-2494 has been discontinued. All remaining material expense was incurred by the end of 2019.

(5) Other expenses include employee and unallocated contractor-related expenses, facility expenses, lab supplies, and miscellaneous expenses.

***Selling, General and Administrative Expenses***

Selling, 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, commercial, operational, finance and human resource functions and other selling, 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 selling, general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our therapeutic candidates. Additionally, if and when we believe regulatory approval of a therapeutic candidate appears likely, to the extent that we are undertaking commercialization of such therapeutic candidate ourselves, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations.

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

Other income (expense), net consists primarily of the re-measurement gain or loss associated with the change in the fair value of our common stock warrant liabilities and interest income earned on cash, cash equivalents and investments.

To estimate the fair value of our liability classified warrants, we use either the Monte Carlo simulation framework, which incorporates future financing events over the remaining life of the warrants to purchase common stock, or for certain re-measurement dates, due to the warrants being deeply in the money, the Black-Scholes option pricing model. We base the estimates in the pricing models, in part, on subjective assumptions, including stock price volatility, risk-free interest rate, dividend yield, and the fair value of the common stock underlying the warrants. The Black-Scholes option pricing model was used at March 31, 2020.

**Critical Accounting Policies and Estimates**

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

During the three months ended March 31, 2020, there have been no material changes to our critical accounting policies as reported in our Annual Report on the Form 10-K for the year ended December 31, 2019. For further information on our critical and other significant accounting policies, including the adoption of ASC 326, 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, 2019.

## Results of Operations

### Comparison of the Three Months Ended March 31, 2020 and 2019

| (in thousands) | Three Months Ended March 31, 2020 | 2019 | Increase (Decrease) |
|---|---|---|---|
| Revenue: | | | |
| Collaboration revenue: | | | |
| Cost-sharing, net | $ 2,824 | $ 2,780 | $ 44 |
| Royalty revenue | 1,520 | — | 1,520 |
| Total revenue (all amounts are with a related party) | 4,344 | 2,780 | 1,564 |
| Costs and expenses: | | | |
| Research and development | 37,663 | 32,771 | 4,892 |
| Selling, general and administrative | 18,253 | 10,814 | 7,439 |
| Total costs and expenses | 55,916 | 43,585 | 12,331 |
| Loss from operations | (51,572) | (40,805) | (10,767) |
| Other income, net | 648 | 2,772 | (2,124) |
| Loss before income taxes | (50,924) | (38,033) | (12,891) |
| Income tax benefit | (15) | (20) | 5 |
| Net loss | $ (50,939) | $ (38,053) | $ (12,886) |

***Revenue.*** We recognized revenue of $4.3 million in the three months ended March 31, 2020, compared to $2.8 million in the same period in 2019. All of the revenue in both periods was derived from the BMS agreements. This $1.5 million increase is primarily related to royalty revenue from REBLOZYL sales recognized in 2020.

***Research and Development Expenses.*** Research and development expenses were $37.7 million in the three months ended March 31, 2020, compared to $32.8 million in the same period in 2019. This $4.9 million increase is primarily related to growth in order to support our wholly-owned therapeutic candidates and preclinical programs and includes:

- an increase in personnel and facilities-related expense of $1.6 million related to increased headcount to support our growth;
- an increase in contract manufacturing and drug supply expense of $6.6 million related to our ongoing clinical and preclinical programs; offset by
- a decrease in external clinical trial expense of $3.6 million due to the discontinuation of our ACE-083 program and wind down of our luspatercept-aamt clinical programs and transfer to BMS.

***Selling, General and Administrative Expenses.*** Selling, general and administrative expenses were $18.3 million in the three months ended March 31, 2020, compared to $10.8 million in the same period in 2019. The $7.5 million increase is primarily due to the following factors:

- an increase in selling expense of $1.9 million to continue supporting the launch of REBLOZYL, our first commercial product approved by the FDA on November 8, 2019; and
- an increase in personnel expense and facilities-related expense of $5.4 million related to increased headcount to support our growth.

***Other Income, Net.*** Other income, net was $0.6 million in the three months ended March 31, 2020, compared to $2.8 million for the same period in 2019. This $2.2 million decrease was primarily due to a $1.4 million increase in the expense associated with marking the common warrant liability to market and a $0.8 million decrease in the interest earned on our investment portfolio as a result of a decrease in interest rates.

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

## Liquidity and Capital Resources

We have incurred losses and cumulative negative cash flows from operations since our inception in June 2003, and as of March 31, 2020, we had an accumulated deficit of $762.3 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 selling, general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of the sale of equity, debt financings or other sources, including potential additional collaborations.

As of March 31, 2020, our operations have been primarily funded by $105.1 million in equity investments from venture investors, $773.8 million from public investors, $154.1 million in equity investments from our collaboration partners and $361.2 million in upfront payments, milestones, and net research and development payments from our collaboration partners.

As of March 31, 2020, we had $415.6 million in cash, cash equivalents and investments. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

**Cash Flows**

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

| | Three Months Ended March 31, | |
|---|---|---|
| **(in thousands)** | **2020** | **2019** |
| Net cash (used in) provided by: | | |
| Operating activities | $ (46,545) | $ (26,308) |
| Investing activities | 77,481 | (152,570) |
| Financing activities | 8,873 | 249,316 |
| Net increase in cash, cash equivalents and restricted cash | $ 39,809 | $ 70,438 |

***Operating Activities***

Net cash used in operating activities was $46.5 million for the three months ended March 31, 2020, compared to $26.3 million during the same period in 2019. Significant factors in this $20.2 million increase include:

- an increase in net loss of $12.9 million due to an increase in operating expenses related to increased headcount and facilities, external expenses for contract manufacturing, consulting, and other external expenses to support our wholly-owned therapeutic programs, as well as expenses for commercial activities for REBLOZYL, offset by an increase in revenue related to royalty revenue associated with sales of REBLOZYL; and

- a net decrease in operating assets and liabilities of $7.5 million, consisting primarily of a decrease in collaboration receivables of $4.1 million and an increase in accrued expenses of $2.6 million.

***Investing Activities***

Net cash provided by investing activities was $77.5 million for the three months ended March 31, 2020, compared to net cash used in investing activities of $152.6 million during the same period in 2019. Net cash provided by and used in investing activities primarily consisted of the following amounts relating to activity within our investment portfolio:

- for the three months ended March 31, 2020, net proceeds from sales and maturities of investments of $77.9 million in connection with managing our investment portfolio to meet our projected cash requirements; and

- for the three months ended March 31, 2019, net purchases of investments of $152.0 million due to the execution of our investment strategy in accordance with our policy as we began to invest the money raised in our January 2019 public offering in marketable securities.

***Financing Activities***

Net cash provided by financing activities was $8.9 million for the three months ended March 31, 2020, compared to $249.3 million during the same period in 2019. Net cash provided by financing activities consisted primarily of the following:

- for the three months ended March 31, 2020, $8.9 million in cash proceeds from the exercise of stock options and the issuance of common stock related to the employee stock purchase plan; and

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

**Operating Capital Requirements**

To date, we have only generated limited revenue from royalties on the sale of our first and only commercial product, REBLOZYL, since receiving our first regulatory approval from the FDA in November 2019. We anticipate that we will continue to generate losses for the foreseeable future as we continue the development of, and seek and obtain regulatory approvals for, sotatercept in the PH field and any future therapeutic candidates, and begin to commercialize any approved products. We are subject to all of the risks inherent in the development of therapeutic candidates, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business.

Based on our current operating plan and projections, we believe that our current cash, cash equivalents and investments, will be sufficient to fund our projected operating requirements until such time as we expect to receive significant royalty revenue from REBLOZYL sales. Independent of the timing or significance of REBLOZYL royalty revenue, however, we may raise additional funds for future development, operations and activities, particularly if there are changes in our operating plan or projections, we add additional programs to our pipeline, or our programs advance in development faster than anticipated.

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

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

- the achievement of milestones and royalties under our agreement with BMS;
- the amount of royalties we receive on sales of REBLOZYL;
- 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 net deferred tax assets of approximately $226.0 million as of December 31, 2019, 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, 2019, we had federal NOL carryforwards of approximately $666.3 million and state NOL carryforwards of $689.8 million available to reduce future taxable income, if any. Of these federal and state NOL carryforwards, $438.0 million and $689.4 million, respectively, will expire at various times through 2039. The federal NOL of $228.3 million and state NOL of $0.4 million generated beginning in 2018 can be carried forward indefinitely. In general, if we experience a greater than 50 percent aggregate change in ownership of certain significant stockholders over a three-year period, or a Section 382 ownership change, utilization of our pre-change NOL carryforwards are subject to an annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended, and similar state laws. Such limitations may result in expiration of a portion of the NOL carryforwards before utilization and may be substantial. If we experience a Section 382 ownership change in connection with our public offerings or as a result of future changes in our stock ownership, some of which changes are outside our control, the tax benefits related to the NOL carryforwards may be limited or lost. For additional information about our taxes, see Note 13 to the financial statements in our Annual Report on Form 10-K for the year ended December 31, 2019.

### Contractual Obligations and Commitments

During the three months ended March 31, 2020, 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, 2019.

### Recent Accounting Pronouncements

For information on recent accounting pronouncements, see Recent Accounting Pronouncements in the notes to the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

### Off-Balance Sheet Arrangements

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

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

We are exposed to market risk related to changes in interest rates. As of March 31, 2020 and December 31, 2019, we had cash, cash equivalents and investments of $415.6 million and $453.8 million, respectively. Our cash equivalents are invested primarily in bank deposits and money market mutual funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Our investments are subject to interest rate risk and could fall in value if market interest rates increase. Due to the duration of our investment portfolio and the low risk profile of our investments, we do not believe an immediate 100 basis point change in interest rates would have a material effect on the fair market value of our portfolio. We have the ability to hold our investments until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

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

## Item 4. Controls and Procedures

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

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities Exchange Act of 1934, or the Exchange Act, is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC’s rules and forms, and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of March 31, 2020, management, with the participation of our Chief Executive Officer and Chief Financial Officer, performed an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures as defined in

Rules 13a-15(e) and 15d-15(e) of the Exchange Act. Our disclosure controls and procedures are designed to ensure that information required to be disclosed in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including the Chief Executive Officer and the Chief Financial Officer, to allow timely decisions regarding required disclosures. Any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objective. Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of March 31, 2020, the design and operation of our disclosure controls and procedures were effective.

**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 March 31, 2020, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II. OTHER INFORMATION

### Item 1. Legal Proceedings

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

### Item 1A. Risk Factors

Except as described below, 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, 2019.

***The COVID-19 pandemic, which began in late 2019 and has spread worldwide, may affect our ability to initiate and complete preclinical studies and conduct our ongoing clinical trials, delay the initiation of planned and future clinical trials, interrupt sales of REBLOZYL® (luspatercept-aamt), disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, this pandemic has caused substantial disruption in the financial markets and economies worldwide which could result in adverse effects on our business and operations.***

As of the date of this Form 10-Q, COVID-19 has been declared by the World Health Organization to be a global pandemic. It has impacted, and is continuing to impact, all aspects of society, including the operation of the healthcare system and other business and economic activity worldwide. The COVID-19 pandemic, and other similar outbreaks of contagious diseases, may adversely impact our business, financial condition and results of operations. For example, we and the third-party clinical trial sites or investigators involved in our current and future clinical trials, may experience significant interruptions or delays as a result of this pandemic, and these could impact the conduct of our clinical trials and our ability to complete them in a timely manner or at all, which in turn could delay and/or negatively impact the regulatory review and approval of our product candidates.

In addition, commercial sales of REBLOZYL may be adversely impacted as a result of developments that have transpired, and may continue to transpire, in response to this pandemic, including the implementation of "shelter-in-place" policies, social distancing and other measures. REBLOZYL is administered via injections in a clinic or hospital setting by a healthcare professional. Treating COVID-19 patients has become the priority for many healthcare facilities and workers, so it has become, and may continue to be, difficult for some of our patients to receive our therapies that are administered by injection. Some patients may also choose to skip injections because they do not want to risk exposure to COVID-19 by leaving their home and entering a healthcare facility.

Further, we have implemented work-at-home policies for employees whose jobs do not require them to be on-site, and our sales force for REBLOZYL has transitioned to online video conferences with healthcare professionals. Increased reliance by us and the companies with which we do business on personnel working from home and through video conferencing may negatively impact productivity, increase cyber security risk, create data accessibility issues, increase the risk for communication disruptions, or otherwise disrupt or delay normal business operations. For our employees whose jobs require them to be on-site, we have taken precautions to avoid the spread of COVID-19 among our employees, but we cannot guarantee our workforce will not face an outbreak that could adversely impact our operations.

The COVID-19 pandemic may also impact the third parties on which we or our partners rely for goods and services in the manufacture of our products, which may negatively impact the ability to continue to manufacture and supply our approved and investigational products, or the ability of third parties in our distribution channels to deliver our approved and investigational products in a timely manner or at all. Further, this pandemic and measures to mitigate the spread of COVID-19 have had, and may continue to have, an adverse effect on global economic conditions, which could have an adverse effect on our business and financial condition, including our ability to obtain financing if needed on favorable terms or at all.

The extent to which the COVID-19 pandemic may impact our business, financial condition and results of operations will depend on the manner in which this pandemic continues to evolve and future developments in response thereto, which are highly uncertain and cannot be predicted with confidence as of the date of this Form 10-Q and which may include, among other things, the ultimate severity and duration of this pandemic; governmental, business or other actions that have been, or will be, taken in response to this pandemic, including restrictions on travel and mobility, business closures and imposition of social distancing measures; impacts of the pandemic on the vendors or distribution channels in our or our partners' supply chain and ability to continue to manufacture our approved and investigational products; impacts of the pandemic on the conduct of our clinical trials, including with respect to enrollment rates, availability of investigators and clinical trial sites or monitoring of data; and impacts of the pandemic on the regulatory agencies with which we interact in the development, review, approval and commercialization of our medicines.

**Item 6. Exhibits**

| Exhibit Number | Description of Exhibit |
|---|---|
| 10.1 | Letter Agreement to Amended and Restated Collaboration, License and Option Agreement between Acceleron Pharma Inc. and Celgene Corporation, dated as of March 10, 2020 |
| 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 | Inline XBRL Taxonomy Extension Schema Document |
| 101.CAL | Inline XBRL Taxonomy Extension Calculation Linkbase Document |
| 101.LAB | Inline XBRL Taxonomy Extension Label Linkbase Document |
| 101.PRE | Inline XBRL Taxonomy Extension Presentation Linkbase Document |
| 101.DEF | Inline XBRL Taxonomy Extension Definition Linkbase Document |
| 104 | Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibits 101) |

## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

**ACCELERON PHARMA INC.**

Date: May 11, 2020

By: /s/ HABIB J. DABLE

Habib J. Dable

*Chief Executive Officer and President*

Date: May 11, 2020

By: /s/ KEVIN F. MCLAUGHLIN

Kevin F. McLaughlin

*Senior Vice President, Chief Financial Officer and Treasurer*

**VIA EMAIL**

March 10, 2020

Celgene Corporation
86 Morris Avenue
Summit, NJ 07901

Re: Clarification of Rights and Obligations under the Sotatercept Agreement (as defined below)

Dear Sir or Madam:

Reference is hereby made to that certain Amended and Restated Collaboration, License and Option Agreement by and between Acceleron Pharma Inc. ("Acceleron") and Celgene Corporation ("Celgene"), dated as of September 18, 2017 (the "Sotatercept Agreement"). Capitalized terms used herein that are not otherwise defined have the meanings set forth in the Sotatercept Agreement.

The parties acknowledge that Acceleron is actively developing the Sotatercept Licensed Compound in the PH Field, and the parties desire to amend certain provisions of the Sotatercept Agreement to allow Acceleron to conduct certain activities relating to the development of the Sotatercept Licensed Compound as set forth below.

Accordingly, the Parties agree as follows:

1. Celgene shall use reasonable efforts to complete the Development Safety Update Report to the Investigational Medicinal Product Dossier required to be submitted to relevant Health Authorities with respect to the Sotatercept Licensed Compound as soon as practicable and, following review of such annual report by Acceleron pursuant to Section 2.3.4 of the Sotatercept Agreement, Celgene shall submit such annual report to the FDA. Following such submission, Celgene shall transfer to Acceleron the responsibility for the Investigational Medicinal Product Dossier at a mutually agreed date.

2. Notwithstanding Section 2.3.5(a) of the Sotatercept Agreement, Acceleron shall maintain the global safety database and clinical study database (the "Databases") for the Sotatercept Licensed Compound and Sotatercept Licensed Product in the Field in the Territory, and Celgene shall provide reasonable assistance to Acceleron in its maintenance of the Databases, at each Party's own expense. Celgene shall transfer to Acceleron such Databases at a mutually agreed date.

3. Celgene shall use reasonable efforts to complete the Investigator Brochure update related to the Sotatercept Licensed Compound as soon as practicable and, following completion of such update, Celgene shall transfer responsibility for such Investigator Brochure to Acceleron as soon as practicable.

4. As soon as practicable following the date of this letter, Celgene shall transfer responsibility for the Material Safety Data Sheet for the Sotatercept Licensed Compound.

5. Celgene hereby provides notice, pursuant to Section 2.4.3 of the Sotatercept Agreement, that: (i) Celgene has elected not to Manufacture Sotatercept Drug Product for the Commercial Supplies of the Sotatercept Licensed Compound and the Sotatercept Licensed Product for the Commercialization of Sotatercept Licensed Products in the PH Field; and (ii) Acceleron is hereby granted an exclusive license under the Celgene Technology and Celgene's interest in the Joint Technology to Manufacture Commercial Supplies of the Sotatercept Licensed Compound and the Sotatercept Licensed Products for the Commercialization of Sotatercept Licensed Products in the PH Field in the Territory.

For the avoidance of doubt, (a) the foregoing does not grant any right or license under any Celgene Technology or Joint Technology to Acceleron to Manufacture Sotatercept Drug Product, the Sotatercept Licensed Compound or the Sotatercept Licensed Product for any purpose other than Commercial Supply of the Sotatercept Licensed Compound and the Sotatercept Licensed Product for the Commercialization of Sotatercept Licensed Product in the PH Field in the Territory; (b) nothing in this letter agreement shall be deemed to preclude, prevent or otherwise restrict Celgene' s right to Manufacture Sotatercept Drug Product in the Celgene Field (for either Clinical Supply or Commercialization purposes); (c) nothing in this letter agreement shall be deemed to designate Acceleron as, or grant Acceleron the right to be, the “Distributing Party” for Sotatercept Licensed Products in the PH Field, as provided in Section 2.6.2 of the Sotatercept Agreement; and (d) nothing in this letter agreement shall be deemed to alter the obligations of the Parties in Section 2.4.3(b) with regard to the technology transfer or the costs associated with such transfer.

Except as expressly amended by this letter agreement, all other terms and conditions of this Sotatercept Agreement shall remain unchanged, and the Sotatercept Agreement shall remain in full force and effect as presently written and the rights, duties, liabilities and obligations of Celgene and Acceleron thereto, as currently constituted, will continue in full force and effect.

Please contact me if you have any questions.

| | |
|---|---|
| Regards, | Acknowledged and Agreed: |
| /s/ Habib Dable | **Celgene Corporation** |
| Habib Dable<br>President and CEO | By: /s/ Nadim Ahmed<br>Name: Nadim Ahmed<br>Title: President, Hematology |

**Exhibit 31.1**

CERTIFICATION OF CHIEF EXECUTIVE OFFICER, ACCELERON PHARMA INC.

I, Habib J. Dable, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Acceleron Pharma Inc.;

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

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

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

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

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

| | |
|---|---|
| May 11, 2020 | /s/ Habib J. Dable |
| Date | Habib J. Dable<br>Chief Executive Officer and President<br>(Principal Executive Officer) |

**Exhibit 31.2**

CERTIFICATION OF CHIEF FINANCIAL OFFICER, ACCELERON PHARMA INC.

I, Kevin F. McLaughlin, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Acceleron Pharma Inc.;

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

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

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

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

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

| | |
|---|---|
| May 11, 2020 | /s/ Kevin F. McLaughlin |
| Date | Kevin F. McLaughlin<br>Senior Vice President, Chief Financial Officer and Treasurer<br>(Principal Financial Officer) |

**Exhibit 32.1**

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

In connection with the Quarterly Report on Form 10-Q of Acceleron Pharma Inc. (the "Company") for the period ended March 31, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his or her knowledge:

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

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

Date: May 11, 2020 By: /s/ Habib J. Dable

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

Date: May 11, 2020 By: /s/ Kevin F. McLaughlin

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