

**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 September 30, 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
(State or other jurisdiction of
incorporation or organization)

2836
(Primary Standard Industrial
Classification Code Number)

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

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

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** **No**

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

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

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

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

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

As of October 31, 2020, there were 60,252,012 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)

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	\$ 729,685	\$ 237,677
Collaboration receivables (all amounts are with a related party)	22,561	8,547
Prepaid expenses and other current assets	8,854	10,000
Short-term investments	152,677	193,692
Total current assets	<u>913,777</u>	<u>449,916</u>
Property and equipment, net	7,621	6,812
Operating lease - right of use asset, net	19,660	23,908
Other assets	1,708	1,793
Long-term investments	4,999	22,477
Total assets	<u>\$ 947,765</u>	<u>\$ 504,906</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 8,861	\$ 2,295
Accrued expenses	21,917	24,895
Operating lease liability - right of use	6,524	6,183
Total current liabilities	<u>37,302</u>	<u>33,373</u>
Operating lease liability - right of use, net of current portion	15,327	20,201
Warrants to purchase common stock	—	1,856
Total liabilities	<u>52,629</u>	<u>55,430</u>
Commitments and contingencies (Note 13)		
Stockholders' equity:		
Common stock, \$0.001 par value: 175,000,000 shares authorized; 60,052,046 and 53,123,567 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	60	53
Additional paid-in capital	1,715,343	1,160,807
Accumulated deficit	(820,042)	(711,407)
Accumulated other comprehensive (loss) income	(225)	23
Total stockholders' equity	<u>895,136</u>	<u>449,476</u>
Total liabilities and stockholders' equity	<u>\$ 947,765</u>	<u>\$ 504,906</u>

See accompanying notes to these condensed consolidated financial statements.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Revenue:				
Collaboration revenue:				
Milestone	\$ —	\$ —	\$ 25,000	\$ 25,000
Cost-sharing, net	3,298	4,208	9,800	9,655
Royalty	19,263	—	31,856	—
Total revenue (all amounts are with a related party)	22,561	4,208	66,656	34,655
Costs and expenses:				
Research and development	40,747	37,630	116,663	105,125
Selling, general and administrative	21,042	15,501	59,705	40,394
Total costs and expenses	61,789	53,131	176,368	145,519
Loss from operations	(39,228)	(48,923)	(109,712)	(110,864)
Other (expense), income net	(6)	3,520	1,108	9,523
Loss before income taxes	(39,234)	(45,403)	(108,604)	(101,341)
Income tax (provision) benefit	(11)	34	(31)	58
Net loss	<u>\$ (39,245)</u>	<u>\$ (45,369)</u>	<u>\$ (108,635)</u>	<u>\$ (101,283)</u>
Other comprehensive (loss) income:				
Net unrealized holding (losses) gains on short-term and long-term investments during the period, net of tax	(142)	(29)	(248)	691
Comprehensive loss	<u>\$ (39,387)</u>	<u>\$ (45,398)</u>	<u>\$ (108,883)</u>	<u>\$ (100,592)</u>
Net loss per share- basic and diluted	\$ (0.66)	\$ (0.86)	\$ (1.95)	\$ (1.94)
Weighted-average number of common shares used in computing net loss per share- basic and diluted	59,640	52,882	55,635	52,239

See accompanying notes to these condensed consolidated financial statements.

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

Three and Nine Months Ended September 30, 2020

	<u>Common Stock</u>					Comprehensive Income (Loss)	Total Stockholders' Equity
	Number of Shares	\$0.001 Par Value	Additional Paid-In Capital	Accumulated Deficit			
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	
Stock-based compensation	—	—	7,140	—	—	7,140	
Exercise of stock options	617,441	1	19,609	—	—	19,610	
Vesting of restricted stock units, net of shares withheld for taxes	29,351	—	(663)	—	—	(663)	
Unrealized gain on available-for-sale securities	—	—	—	—	161	161	
Net loss	—	—	—	(18,451)	—	(18,451)	
Balance at June 30, 2020	54,166,712	54	1,202,445	(780,797)	(83)	421,619	
Stock-based compensation	—	—	7,986	—	—	7,986	
Issuance of common stock net of expense \$505	5,594,593	6	492,408	—	—	492,414	
Exercise of stock options	229,763	—	8,284	—	—	8,284	
Vesting of restricted stock units, net of shares withheld for taxes	10,879	—	(313)	—	—	(313)	
Issuance of common stock related to ESPP	13,026	—	804	—	—	804	
Net exercise of warrants to purchase common stock	37,073	—	3,729	—	—	3,729	
Unrealized loss on available-for-sale securities	—	—	—	—	(142)	(142)	
Net loss	—	—	—	(39,245)	—	(39,245)	
Balance at September 30, 2020	60,052,046	\$ 60	\$ 1,715,343	\$ (820,042)	\$ (225)	\$ 895,136	

Three and Nine Months Ended September 30, 2019

	Common Stock					Comprehensive Loss	Total Stockholders' Equity
	Number of Shares	\$0.001 Par Value	Additional Paid-In Capital	Accumulated Deficit			
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	
Stock-based compensation	—	—	5,012	—	—	5,012	
Exercise of stock options	64,174	—	1,760	—	—	1,760	
Vesting of restricted stock units, net of shares withheld for taxes	146,162	—	—	—	—	—	
Unrealized gain on available-for-sale securities, net of tax	—	—	—	—	452	452	
Net loss	—	—	—	(17,862)	—	(17,862)	
Balance at June 30, 2019	52,752,854	53	1,142,148	(642,464)	160	499,897	
Stock-based compensation	—	—	5,306	—	—	5,306	
Exercise of stock options	156,394	1	4,743	—	—	4,744	
Vesting of restricted stock units, net	104,217	—	(252)	—	—	(252)	
Issuance of common stock related to ESPP	13,598	—	504	—	—	504	
Unrealized loss on available-for-sale securities, net of tax	—	—	—	—	(29)	(29)	
Net loss	—	—	—	(45,369)	—	(45,369)	
Balance at September 30, 2019	53,027,063	\$ 54	\$ 1,152,449	(687,833)	\$ 131	\$ 464,801	

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

	Nine Months Ended September 30,	
	2020	2019
Operating Activities		
Net loss	\$ (108,635)	\$ (101,283)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	2,841	2,932
Stock-based compensation	21,805	17,310
Other non-cash items	1,552	(75)
Changes in assets and liabilities:		
Prepaid expenses and other assets	1,231	(3,731)
Collaboration receivables (all amounts are with a related party)	(14,014)	164
Non-cash lease expense	4,249	3,804
Accounts payable	6,315	1,435
Accrued expenses	(3,025)	5,132
Operating lease obligations	(4,533)	(3,214)
Other changes in operating assets and liabilities	17	(27)
Net cash used in operating activities	(92,197)	(77,553)
Investing Activities		
Purchases of investments	(161,741)	(379,096)
Proceeds from sales and maturities of investments	220,240	228,258
Purchases of property and equipment	(3,303)	(2,166)
Net cash provided by (used in) investing activities	55,196	(153,004)
Financing Activities		
Proceeds from issuance of common stock from public offering, net of issuance costs	492,414	248,130
Net proceeds from exercises and vesting of stock awards, and ESPP contributions	36,595	7,917
Net cash provided by financing activities	529,009	256,047
Net increase in cash, cash equivalents and restricted cash	492,008	25,490
Cash, cash equivalents and restricted cash at beginning of period	239,274	145,649
Cash, cash equivalents and restricted cash at end of period	\$ 731,282	\$ 171,139
Supplemental Disclosure of Non-Cash Investing and Financing Activities:		
Purchase of property and equipment included in accounts payable and accrued expenses	\$ 281	\$ 512
Reclassification of warrant liability to additional paid-in capital	\$ 3,729	\$ —

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 or successfully commercializes its products, 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 September 30, 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 September 30, 2020, the results of its operations for the three and nine months ended September 30, 2020 and 2019, and its cash flows for the nine months ended September 30, 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 and nine months ended September 30, 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 September 30, 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 September 30, 2020 (in thousands):

	Less than 12 months	
	Fair Value	Unrealized Losses
Corporate obligations	18,756	(1)
U.S. government agency securities	101,674	(16)
Agency bond	4,999	(1)
Total available-for sale securities in an unrealized loss position	\$ 125,429	\$ (18)

There were no securities in an unrealized loss position for greater than 12 months as of September 30, 2020. The unrealized losses on the Company's available-for-sale securities 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 September 30, 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 September 30, 2020 and December 31, 2019 (in thousands):

	September 30, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash and cash equivalents due in 90 days or less	\$ 729,686	\$ —	\$ (1)	\$ 729,685
Available-for-sale securities:				
Corporate obligations	39,162	57	(1)	39,218
U.S. Treasury securities	113,220	8	(16)	113,212
Certificates of deposit	245	2	—	247
Mortgage and other asset backed securities	5,000	—	(1)	4,999
Total available-for-sale securities (1)	<u>\$ 157,627</u>	<u>\$ 67</u>	<u>\$ (18)</u>	<u>\$ 157,676</u>
Total cash, cash equivalents and available-for-sale securities	<u>\$ 887,313</u>	<u>\$ 67</u>	<u>\$ (19)</u>	<u>\$ 887,361</u>
	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 (1)	<u>\$ 215,872</u>	<u>\$ 325</u>	<u>\$ (28)</u>	<u>\$ 216,169</u>
Total cash, cash equivalents and available-for-sale securities	<u>\$ 453,549</u>	<u>\$ 325</u>	<u>\$ (28)</u>	<u>\$ 453,846</u>

(1) All available-for-sale securities mature within two and three years as of September 30, 2020 and December 31, 2019, respectively.

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):

	September 30,	
	2020	2019
Cash and cash equivalents	\$ 729,685	\$ 169,542
Restricted cash	1,597	1,597
Total cash, cash equivalents and restricted cash shown in the statement of cash flows	<u>\$ 731,282</u>	<u>\$ 171,139</u>

As of September 30, 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. Restricted cash is included within other assets in our condensed consolidated balance sheet.

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 September 30, 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 September 30, 2020 and December 31, 2019 (in thousands):

	September 30, 2020			Total
	Quoted Prices in Active Markets for Identical Items (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Assets:				
Money market funds	\$ 186,379	\$ —	\$ —	\$ 186,379
Corporate obligations	—	43,336	—	43,336
U.S. Treasury securities	—	123,210	—	123,210
Certificates of deposit	—	247	—	247
Mortgage and other asset backed securities	—	4,999	—	4,999
Total assets	\$ 186,379	\$ 171,792	\$ —	\$ 358,171

	December 31, 2019			Total
	Quoted Prices in Active Markets for Identical Items (Level 1)	Significant other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Assets:				
Money market funds	\$ 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 nine months ended September 30, 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):

	Nine Months Ended September 30,	
	2020	2019
Beginning balance	\$ 1,856	\$ 1,491
Change in fair value	1,873	(165)
Settlement	(3,729)	—
Ending balance	\$ —	\$ 1,326

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. All remaining outstanding warrants were remeasured using the Black-Scholes model upon their expiration on July 9, 2020. At September 30, 2020, there were no warrants outstanding.

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 September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Outstanding stock options	3,546	3,920	3,546	3,920
Common stock warrants	—	39	—	39
Shares issuable under employee stock purchase plan	19	23	19	23
Outstanding restricted stock units (1)	572	366	572	366
	4,137	4,348	4,137	4,348

(1) This balance is comprised of both the restricted stock units and performance-based restricted stock units described in Note 16.

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 September 30, 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	Expiration	Balance Sheet Classification	
	September 30, 2020	December 31, 2019			September 30, 2020	December 31, 2019
Warrants to purchase common stock	—	39	\$ 5.88	July 9, 2020	N/A (1)	Liability

(1) All remaining outstanding warrants were automatically cashless exercised in full upon their expiration on July 9, 2020.

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 September 30, 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 September 30, 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 were completed and all remaining potential milestone payments were fully constrained as they related to future regulatory events that were outside of the Company's control, and therefore the risk of significant reversal in the amount of cumulative revenue had not been resolved. As of September 30, 2020, the last remaining regulatory milestone payment for REBLOZYL would be \$20.0 million and would result from approval by a regulatory authority in Asia (as defined in the REBLOZYL Agreement) of a Biologics License Application (BLA) or equivalent for luspatercept-aamt in either myelodysplastic syndromes or beta-thalassemia. 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 September 30, 2020, the Company cannot determine if it is probable that a regulatory agency will approve the applications.

In June 2020, the European Commission, which has the authority to approve medicines for the European Union, approved REBLOZYL based on the recommendation of the European Medicines Agency, or EMA, 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. As a result, the \$25.0 million milestone from the EMA approval of a BLA or equivalent for REBLOZYL was no longer constrained. As the Company did not have any remaining performance obligations under the agreement with BMS, the full \$25.0 million was recognized as revenue during the three months ended June 30, 2020.

Through September 30, 2020, under all BMS arrangements, the Company has received net cost-share payments, milestones, and royalties of \$234.3 million and \$44.9 million for REBLOZYL and sotatercept, respectively. The Company recorded net collaboration revenue of \$22.6 million and \$4.2 million during the three months ended September 30, 2020 and 2019, respectively, and \$66.7 million and \$34.7 million during the nine months ended September 30, 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-sublicensable 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 September 30, 2020 and 2019, the Company expensed \$1.0 million and zero, respectively, and during the nine months ended September 30, 2020 and 2019, the Company expensed \$3.4 million and \$1.6 million, respectively, of milestones, fees and royalties. 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 September 30, 2020 and 2019, the Company expensed \$0.1 million and \$0.1 million, respectively, and during the nine months ended September 30, 2020 and 2019, the Company expensed \$0.6 million and \$2.1 million, respectively, of milestones and fees, 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, which was expensed to research and development. 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 September 30, 2020 and 2019, the Company expensed \$0.6 million and

zero, respectively, and during the nine months ended September 30, 2020 and 2019, the Company expensed \$1.7 million and zero, respectively, of fees, which is recorded as research and development expense.

15. Stockholders' Equity

On July 6, 2020, the Company completed the sale in an underwritten public offering of 5,594,593 shares of common stock, including 729,729 shares of common stock sold pursuant to the underwriter's full exercise of their option to purchase additional shares, at a public offering price of \$92.50 per share, resulting in net proceeds to the Company of \$492.4 million.

16. 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 and nine months ended September 30, 2020 and 2019, respectively, as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	\$ 3,794	\$ 2,107	\$ 10,194	\$ 7,610
Selling, general and administrative	4,192	3,199	11,611	9,700
	<u>\$ 7,986</u>	<u>\$ 5,306</u>	<u>\$ 21,805</u>	<u>\$ 17,310</u>

Stock Options

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Expected volatility	57.7 %	57.4 %	55.6 %	58.7 %
Expected term (in years)	6.0	6.0	6.0	6.0
Risk-free interest rate	0.4 %	1.6 %	1.4 %	2.4 %
Expected dividend yield	— %	— %	— %	— %

The following table summarizes the stock option activity under the Company's stock option plans during the nine months ended September 30, 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	1,041	\$ 62.88		
Exercised	(1,143)	\$ 31.83		
Canceled or forfeited	(172)	\$ 42.71		
Outstanding at September 30, 2020	<u>3,546</u>	\$ 45.19	7.42	\$ 238,802
Exercisable at September 30, 2020	<u>1,843</u>	\$ 37.22	6.22	\$ 138,783

(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 September 30, 2020.

The aggregate intrinsic value of options exercised during the nine months ended September 30, 2020 was \$67.7 million.

As of September 30, 2020, there was \$44.1 million of unrecognized compensation expense related to unvested stock options that is expected to be recognized over a weighted-average period of 2.61 years.

Restricted Stock Units

The following table summarizes the restricted stock unit (RSU) activity under the 2013 Plan during the nine months ended September 30, 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	275	67.45
Vested	(137)	38.64
Forfeited	(38)	42.80
Unvested balance at September 30, 2020	497	\$ 54.68

As of September 30, 2020, there was approximately \$20.7 million of related unrecognized compensation cost, which the Company expects to recognize over a remaining weighted-average period of 1.79 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 by December 31, 2022. As of September 30, 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 September 30, 2020, no related compensation cost had been recognized. The following table summarizes PSU activity under the 2013 Plan during the nine months ended September 30, 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 September 30, 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 September 30, 2020, there was approximately \$4.0 million of related unrecognized compensation cost. Depending on the actual number of awards earned, the actual expense recognized could range between 0% and 200% of this amount.

17. 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.

18. Related Party Transactions**BMS**

BMS owned 10.9% and 12.0% of the Company's fully diluted equity as of September 30, 2020 and December 31, 2019, respectively. Refer to Note 14 for additional information regarding this collaboration arrangement.

During the three and nine months ended September 30, 2020 and 2019, all revenue recognized by the Company was recognized under the BMS collaboration arrangement.

19. Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The Company has evaluated all subsequent events and determined that there are no material recognized or unrecognized subsequent events requiring disclosure except as described below.

On October 16, 2020, the Company entered into an amendment to the lease agreement for its office space at 125 Sidney Street to extend the lease term to May 31, 2026. Under the terms of this amendment, the base rental rate ranges from \$65.0 thousand to \$71.8 thousand per month, for a total commitment of \$4.1 million during the extended lease term. The Company is currently evaluating the impact the amendment will have on its consolidated financial statements and related disclosures.

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'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 and Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2020 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. In June 2020, the European Commission, which has the authority to approve medicines for the European Union, approved REBLOZYL based on the recommendation of the European Medicines Agency, or EMA, 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. In September 2020, Health Canada approved REBLOZYL for the treatment of adult patients with transfusion-dependent anemia associated with beta-thalassemia.

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 or after 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 first quarter of 2021 the Phase 3 INDEPENDENCE study in patients with myelofibrosis-associated anemia who are being treated with JAK inhibitor therapy and require RBC transfusions.

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 \$100.0 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 June 2020, we presented topline results of the PULSAR Phase 2 trial of sotatercept in patients with PAH on stable background PAH-specific therapies during the "Breaking News: Clinical Trials in Pulmonary Medicine" session of the American Thoracic Society, or ATS, 2020 Virtual Conference. Study investigators reported that the trial met its primary endpoint, pulmonary vascular resistance, and its key secondary endpoint, six-minute walk distance, and showed concordance of results across multiple additional endpoints and regardless of baseline characteristics. Sotatercept was generally well tolerated in the trial and adverse events observed in the study were generally consistent with previously published data on sotatercept in clinical trials in other patient populations. The 18-month extension period of the PULSAR trial is ongoing and we plan to initiate our registrational Phase 3 trial, the STELLAR trial, in patients with PAH by year-end 2020. We also plan to initiate an early intervention Phase 3 trial, the HYPERION trial, and a later intervention Phase 3 trial, the ZENITH trial, in expanded PAH patient populations in mid-2021.

We are currently enrolling an exploratory study called SPECTRA to provide us with greater understanding of sotatercept's potential impact on PAH, with preliminary interim results expected to be presented in November 2020 at the virtual 2020 American Heart Association Scientific Sessions, and additional results expected in the first half of 2021. We also previously 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 September 30, 2020, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$1.3 billion from public investors, \$164.1 million in equity investments from our collaboration partners and \$409.4 million in upfront payments, milestones, royalties, and net research and development payments from our collaboration partners.

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;
- prepare for the potential launch and commercialization of sotatercept in the PH field;
- 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 royalty revenue from the sale of our first and only commercial product, REBLOZYL, and potentially from the sale of equity, debt financings or other sources, including additional collaborations. However, we may not generate sufficient royalty revenue and 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 generate significant revenue or 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 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 our expenses to increase and to incur losses as we continue the development of, and seek regulatory approvals for, sotatercept in the PH field and any future therapeutic candidates, and potentially begin to commercialize any additional 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 and Quarterly Report on Form 10-Q for the quarter ended March 31, 2020.

Financial Operations Overview

Impact of COVID-19 on our Business

A novel strain of coronavirus (COVID-19) was declared a global pandemic by the World Health Organization (WHO) in March 2020 and has caused an economic downturn on a global scale, as well as significant volatility in the financial markets. As of September 30, 2020, we have not experienced a significant financial or supply chain impact directly related to the pandemic, but have experienced some disruptions to clinical operations with regard to timelines to complete patient enrollment in our ongoing Phase 2 SPECTRA trial, and may experience similar disruptions in our planned Phase 3 sotatercept clinical trials. We have also experienced disruptions in our commercialization efforts for REBLOZYL with regard to customer engagement and in-person promotion. In addition, as various geographies in the United States and worldwide adapt to surging COVID-19 infections, we may experience additional setbacks to our operations that could have a material impact on our business.

For a discussion of the risks presented by the COVID-19 pandemic to our results, see Risk Factors in Part II, Item 1A of the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2020.

Revenue

Collaboration Revenue

Our revenue to date has been predominantly derived from collaboration revenue, which includes license and milestone revenue, cost-sharing revenue, and royalties, generated through collaboration and license agreements with partners for the development and commercialization of our therapeutic candidates. Cost-sharing revenue represents amounts reimbursed by our collaboration partners for expenses incurred by us for research and development activities and co-promotion activities under our collaboration agreements. Cost-sharing revenue is recognized in the period that the related activities are performed. Royalty revenue is recognized in the period that 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 September 30, 2020, we have incurred \$929.3 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 and nine months ended September 30, 2020 and 2019 are as follows:

(in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Luspatercept-aamt (1)	\$ 808	\$ 1,027	\$ 2,154	\$ 3,288
Sotatercept (2)	13,914	3,762	31,638	11,900
ACE-083 (3)	569	11,064	7,093	21,614
Total direct research and development expenses	15,291	15,853	40,885	36,802
Other expenses (4)	25,456	21,777	75,778	68,323
Total research and development expenses	\$ 40,747	\$ 37,630	\$ 116,663	\$ 105,125

(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) Other expenses include employee and unallocated contractor-related expenses, facility expenses, lab supplies, and miscellaneous research expenses, including expenses associated with preclinical and other development programs.

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. Selling, general and administrative expenses also include directors' fees, professional fees for accounting and legal services, other miscellaneous costs associated with supporting our sales, marketing and investor relations activities, and allocated facilities, depreciation, and other expenses, such as rent and maintenance of facilities, insurance and other supplies.

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. All remaining outstanding warrants were remeasured using the Black-Scholes model upon their expiration on July 9, 2020. At September 30, 2020, there were no warrants outstanding.

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 and nine months ended September 30, 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 September 30, 2020 and 2019

(in thousands)	Three Months Ended		Increase (Decrease)
	September 30,		
	2020	2019	
Revenue:			
Collaboration revenue:			
Cost-sharing, net	3,298	4,208	(910)
Royalty	19,263	—	19,263
Total revenue (all amounts are with a related party)	22,561	4,208	18,353
Costs and expenses:			
Research and development	40,747	37,630	3,117
Selling, general and administrative	21,042	15,501	5,541
Total costs and expenses	61,789	53,131	8,658
Loss from operations	(39,228)	(48,923)	9,695
Other (expense) income, net	(6)	3,520	(3,526)
Loss before income taxes	(39,234)	(45,403)	6,169
Income tax (provision) benefit	(11)	34	(45)
Net loss	\$ (39,245)	\$ (45,369)	\$ 6,124

Revenue. We recognized revenue of \$22.6 million in the three months ended September 30, 2020, compared to \$4.2 million in the same period in 2019. All of the revenue in both periods was derived from the BMS agreements. This \$18.4 million increase is primarily related to royalty revenue from REBLOZYL sales recognized in 2020 of \$19.3 million, offset by decreased cost-sharing revenue due to a decrease in reimbursable commercial fees, trial management fees, and development costs of \$0.9 million.

Research and Development Expenses. Research and development expenses were \$40.7 million in the three months ended September 30, 2020, compared to \$37.6 million in the same period in 2019. This \$3.1 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 \$3.6 million related to increased headcount to support our growth;
- an increase in contract manufacturing, drug supply, and toxicology expenses of \$0.1 million related to our ongoing clinical and preclinical programs; and
- an increase in miscellaneous research expense of \$1.1 million; offset by
- a decrease in external clinical trial expense of \$1.7 million due to the discontinuation of our ACE-083 program, as well as decreased clinical activities for the luspatercept Phase 2 clinical trials.

Selling, General and Administrative Expenses. Selling, general and administrative expenses were \$21.0 million in the three months ended September 30, 2020, compared to \$15.5 million in the same period in 2019. The \$5.5 million increase is primarily due to the following factors:

- an increase in personnel expense and facilities-related expense of \$2.1 million related to increased headcount to support our growth;
- an increase in selling expense of \$2.1 million to support the launch of REBLOZYL, which was approved for its second indication by the FDA in April 2020, as well as market research and market development activities for sotatercept; and
- an increase in other miscellaneous expenses of \$1.3 million.

Other Income, Net. Other expense, net was \$6.0 thousand in the three months ended September 30, 2020, compared to other income, net of \$3.5 million for the same period in 2019. This \$3.5 million decrease was primarily due to a \$0.6 million decrease in sublease income, a \$0.3 million decrease in the expense associated with marking the common warrant liability to market upon auto exercise in July 2020, and a \$2.5 million decrease in the interest earned on our investment portfolio as a result of a decrease in the balance of our investments.

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

Comparison of the Nine Months Ended September 30, 2020 and 2019

(in thousands)	Nine Months Ended		Increase (Decrease)
	September 30,		
	2020	2019	
Revenue:			
Collaboration revenue:			
Milestone	\$ 25,000	\$ 25,000	\$ —
Cost-sharing, net	9,800	9,655	145
Royalty	31,856	—	31,856
Total revenue (all amounts are with a related party)	66,656	34,655	32,001
Costs and expenses:			
Research and development	116,663	105,125	11,538
Selling, general and administrative	59,705	40,394	19,311
Total costs and expenses	176,368	145,519	30,849
Loss from operations	(109,712)	(110,864)	1,152
Other income, net	1,108	9,523	(8,415)
Loss before income taxes	(108,604)	(101,341)	(7,263)
Income tax (provision) benefit	(31)	58	(89)
Net loss	\$ (108,635)	\$ (101,283)	\$ (7,352)

Revenue. We recognized revenue of \$66.7 million in the nine months ended September 30, 2020, compared to \$34.7 million in the same period in 2019. All of the revenue in both periods was derived from the BMS agreements. This \$32.0 million increase is primarily related to royalty revenue from REBLOZYL sales recognized in 2020.

Research and Development Expenses. Research and development expenses were \$116.7 million in the nine months ended September 30, 2020, compared to \$105.1 million in the same period in 2019. This \$11.6 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 \$7.5 million related to increased headcount to support our growth;
- an increase in contract manufacturing, drug supply and toxicology expenses of \$10.9 million related to our ongoing clinical and preclinical programs; and
- an increase in miscellaneous research expense of \$2.6 million; offset by
- a decrease in external clinical trial expense of \$9.4 million due to the discontinuation of our ACE-083 program, as well as decreased clinical activities for the luspatercept Phase 2 clinical trials.

Selling, General and Administrative Expenses. Selling, general and administrative expenses were \$59.7 million in the nine months ended September 30, 2020, compared to \$40.4 million in the same period in 2019. The \$19.3 million increase is primarily due to the following factors:

- an increase in personnel expense and facilities-related expense of \$11.9 million related to increased headcount to support our growth;

- an increase in selling expense of \$5.5 million to continue supporting the launch of REBLOZYL, which was approved for its second indication by the FDA in April 2020, as well as market research and market development activities for sotatercept; and
- an increase in other miscellaneous expenses of \$1.9 million.

Other Income, Net. Other income, net was \$1.1 million in the nine months ended September 30, 2020, compared to \$9.5 million for the same period in 2019. This \$8.4 million decrease was primarily due to a \$2.0 million decrease in the expense associated with marking the common warrant liability to market, a \$0.7 million decrease in sublease income, and a \$5.6 million decrease in the interest earned on our investment portfolio as a result of a decrease in the balance of our investments.

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 September 30, 2020, we had an accumulated deficit of \$820.0 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 may 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 September 30, 2020, our operations have been primarily funded by \$105.1 million in equity investments from venture investors, \$1.3 billion from public investors, \$164.1 million in equity investments from our collaboration partners, and \$409.4 million in upfront payments, milestones, royalties, and net research and development payments from our collaboration partners.

As of September 30, 2020, we had \$887.4 million in cash, cash equivalents and investments. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

Cash Flows

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

(in thousands)	Nine Months Ended September 30,	
	2020	2019
Net cash (used in) provided by:		
Operating activities	\$ (92,197)	\$ (77,553)
Investing activities	55,196	(153,004)
Financing activities	529,009	256,047
Net increase in cash, cash equivalents and restricted cash	\$ 492,008	\$ 25,490

Operating Activities

Net cash used in operating activities was \$92.2 million for the nine months ended September 30, 2020, compared to \$77.6 million during the same period in 2019. Significant factors in this \$14.6 million increase include:

- an increase in net loss of \$7.4 million primarily 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 royalty revenue associated with sales of REBLOZYL;
- a net increase in operating assets and liabilities of \$13.3 million, consisting primarily of an increase in collaboration receivables of \$14.2 million; and
- a net increase in other non-cash expenses of \$6.0 million largely related to an increase in stock-based compensation expense and expense associated with the remeasurement of the fair value of the common warrant liability upon auto-exercise in July 2020.

Investing Activities

Net cash provided by investing activities was \$55.2 million for the nine months ended September 30, 2020, compared to net cash used in investing activities of \$153.0 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 nine months ended September 30, 2020, proceeds from maturities of investments of \$58.5 million net of purchases, in connection with managing our investment portfolio to meet our projected cash requirements; and
- for the nine months ended September 30, 2019, purchases of investments of \$150.8 million net of maturities, 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 \$529.0 million for the nine months ended September 30, 2020, compared to \$256.0 million during the same period in 2019. Net cash provided by financing activities consisted primarily of the following:

- for the nine months ended September 30, 2020, \$492.4 million in net cash proceeds from our July 2020 public offering and the underwriter's full exercise of their option to purchase additional shares, as well as \$36.6 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 nine months ended September 30, 2019, \$248.1 million in net cash proceeds from our January 2019 public offering and the underwriter's full exercise of their option to purchase additional shares, as well as \$7.9 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 expect our expenses to increase and to incur losses as we continue the development of, and seek 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, along with the expected royalty revenue from REBLOZYL sales, will be sufficient to fund our projected operating requirements for the foreseeable future.

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

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

- the achievement of milestones under our agreement with 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 costs of preparing for the potential launch and commercialization of sotatercept or our other 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 September 30, 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 Note 11 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 September 30, 2020 and December 31, 2019, we had cash, cash equivalents and investments of \$887.4 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 September 30, 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 September 30, 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 September 30, 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

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 and the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2020.

Item 6. Exhibits

<u>Exhibit Number</u>	<u>Description of Exhibit</u>
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: November 5, 2020

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

Date: November 5, 2020

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

CERTIFICATION OF CHIEF EXECUTIVE OFFICER, ACCELERON PHARMA INC.

I, Habib J. Dable, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Acceleron Pharma Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f))) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent function):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

November 5, 2020

Date

/s/ Habib J. Dable

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

CERTIFICATION OF CHIEF FINANCIAL OFFICER, ACCELERON PHARMA INC.

I, Kevin F. McLaughlin, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Acceleron Pharma Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f))) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent function):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

November 5, 2020

Date

/s/ Kevin F. McLaughlin

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

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

In connection with the Quarterly Report on Form 10-Q of Acceleron Pharma Inc. (the "Company") for the period ended September 30, 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: November 5, 2020

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

Date: November 5, 2020

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