

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: 001-36183

Eiger BioPharmaceuticals, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware
(State or Other Jurisdiction of
Incorporation or Organization)

2155 Park Boulevard
Palo Alto, CA
(Address of Principal Executive Offices)

33-0971591
(I.R.S. Employer
Identification No.)

94306
(Zip Code)

(650) 272-6138

(Registrant's Telephone Number, Including Area Code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock (par value \$0.001 per share)	EIGR	The Nasdaq Stock Market LLC

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 (§232.405 of this chapter) 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 type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input checked="" 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 November 2, 2020, the number of outstanding shares of the registrant's common stock, par value \$0.001 per share, was 32,531,693.

EIGER BIOPHARMACEUTICALS, INC.
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In this Quarterly Report on Form 10-Q, "we," "our," "us," "Eiger," and "the Company" refer to Eiger BioPharmaceuticals, Inc. Eiger, Eiger BioPharmaceuticals, the Eiger logo and other trade names, trademarks or service marks of Eiger are the property of Eiger BioPharmaceuticals, Inc. This Quarterly Report on Form 10-Q contains references to our trademarks and to trademarks belonging to other entities. Trade names, trademarks and service marks of other companies appearing in this Quarterly Report on Form 10-Q are the property of their respective holders. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

Eiger BioPharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(In thousands)

	September 30, 2020 (Unaudited)	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	\$ 51,993	\$ 39,373
Debt securities, available-for-sale	73,341	55,621
Prepaid expenses and other current assets	9,608	5,390
Total current assets	134,942	100,384
Property and equipment, net	670	590
Operating lease right-of-use assets	1,300	1,654
Other assets	3,781	2,511
Total assets	<u>\$ 140,693</u>	<u>\$ 105,139</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 8,250	\$ 6,414
Accrued liabilities	7,062	10,001
Current portion of operating lease liabilities	565	534
Current portion of long-term debt, net	5,783	—
Total current liabilities	21,660	16,949
Long-term debt, net	25,200	30,390
Operating lease liabilities	883	1,320
Total liabilities	47,743	48,659
Stockholders' equity:		
Common stock	32	24
Additional paid-in capital	380,596	297,863
Accumulated other comprehensive income	16	42
Accumulated deficit	(287,694)	(241,449)
Total stockholders' equity	92,950	56,480
Total liabilities and stockholders' equity	<u>\$ 140,693</u>	<u>\$ 105,139</u>

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Eiger BioPharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	\$ 9,810	\$ 14,059	\$ 29,045	\$ 39,863
General and administrative	5,027	4,247	15,141	12,529
Total operating expenses	14,837	18,306	44,186	52,392
Loss from operations	(14,837)	(18,306)	(44,186)	(52,392)
Interest expense	(906)	(884)	(2,681)	(2,518)
Interest income	76	585	629	1,598
Other expense, net	(13)	(11)	(7)	(20)
Net loss	\$ (15,680)	\$ (18,616)	\$ (46,245)	\$ (53,332)
Net loss per common share, basic and diluted	\$ (0.52)	\$ (0.76)	\$ (1.74)	\$ (2.40)
Weighted-average common shares outstanding, basic and diluted	29,879,135	24,437,451	26,639,201	22,261,715

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Eiger BioPharmaceuticals, Inc.
Condensed Consolidated Statements of Comprehensive Loss
(Unaudited)
(In thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Net loss	\$ (15,680)	\$ (18,616)	\$ (46,245)	\$ (53,332)
Other comprehensive (loss) gain:				
Unrealized (loss) gain on available-for-sale debt securities, net	(29)	38	(26)	68
Comprehensive loss	<u>\$ (15,709)</u>	<u>\$ (18,578)</u>	<u>\$ (46,271)</u>	<u>\$ (53,264)</u>

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Eiger BioPharmaceuticals, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(Unaudited)
(In thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2019	24,523,381	\$ 24	\$ 297,863	\$ 42	\$ (241,449)	\$ 56,480
Issuance of common stock upon exercise of stock options	2,895	—	30	—	—	30
Vesting of common stock issued under Product Development Agreement	—	—	53	—	—	53
Issuance of common stock upon ESPP purchase	11,332	—	83	—	—	83
Issuance of common stock upon offering at-the-market, net of issuance costs of \$221	32,751	—	191	—	—	191
Stock-based compensation expense	—	—	1,629	—	—	1,629
Unrealized loss on debt securities, net	—	—	—	(6)	—	(6)
Net loss	—	—	—	—	(15,239)	(15,239)
Balance at March 31, 2020	24,570,359	24	299,849	36	(256,688)	43,221
Issuance of common stock upon offering at-the-market, net of issuance costs of \$786	2,612,476	3	28,577	—	—	28,580
Issuance of common stock upon exercise of stock options	58,805	—	490	—	—	490
Vesting of common stock issued under Product Development Agreement	—	—	55	—	—	55
Stock-based compensation expense	—	—	1,462	—	—	1,462
Unrealized gain on debt securities, net	—	—	—	9	—	9
Net loss	—	—	—	—	(15,326)	(15,326)
Balance at June 30, 2020	27,241,640	27	330,433	45	(272,014)	58,491
Vesting of common stock issued under Product Development Agreement	—	—	56	—	—	56
Issuance of common stock upon ESPP purchase	14,313	—	103	—	—	103
Issuance of common stock upon offering at-the-market, net of issuance costs of \$1,575	4,660,086	5	48,559	—	—	48,564
Stock-based compensation expense	—	—	1,445	—	—	1,445
Unrealized loss on debt securities, net	—	—	—	(29)	—	(29)
Net loss	—	—	—	—	(15,680)	(15,680)
Balance at September 30, 2020	31,916,039	\$ 32	\$ 380,596	\$ 16	\$ (287,694)	\$ 92,950

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Eiger BioPharmaceuticals, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(Unaudited)
(In thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2018	19,211,759	\$ 19	\$ 237,795	\$ (25)	\$ (171,197)	\$ 66,592
Issuance of common stock upon exercise of stock options	41,546	—	65	—	—	65
Vesting of common stock issued under Product Development Agreement	—	—	56	—	—	56
Issuance of common stock upon ESPP purchase	7,138	—	59	—	—	59
Stock-based compensation expense	—	—	1,195	—	—	1,195
Unrealized gain on debt securities, net	—	—	—	30	—	30
Net loss	—	—	—	—	(17,189)	(17,189)
Balance at March 31, 2019	19,260,443	19	239,170	5	(188,386)	50,808
Issuance of common stock upon exercise of stock options	10,304	—	62	—	—	62
Vesting of common stock issued under Product Development Agreement	—	—	55	—	—	55
Issuance of common stock upon public offering, net of \$3,731 of issuance costs	5,175,000	5	53,189	—	—	53,194
Stock-based compensation expense	—	—	1,487	—	—	1,487
Net loss	—	—	—	—	(17,527)	(17,527)
Balance at June 30, 2019	24,445,747	24	293,963	5	(205,913)	88,079
Issuance of common stock upon exercise of stock options	39,875	—	379	—	—	379
Vesting of common stock issued under Product Development Agreement	—	—	59	—	—	59
Issuance of common stock upon ESPP purchase	8,563	—	71	—	—	71
Stock-based compensation expense	—	—	1,649	—	—	1,649
Unrealized gain on debt securities, net	—	—	—	38	—	38
Net loss	—	—	—	—	(18,616)	(18,616)
Balance at September 30, 2019	24,494,185	\$ 24	\$ 296,121	\$ 43	\$ (224,529)	\$ 71,659

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Eiger BioPharmaceuticals, Inc.
Condensed Consolidated Statements of Cash Flow
(Unaudited)
(In thousands)

	Nine Months Ended September 30,	
	2020	2019
Operating activities		
Net loss	\$ (46,245)	\$ (53,332)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	112	41
Amortization of debt securities discounts	(33)	(390)
Non-cash interest expense	593	535
Amortization of operating lease right-of-use assets	354	299
Common stock issued under Product Development Agreement	164	170
Stock-based compensation	4,536	4,331
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(4,418)	(2,967)
Other assets	(1,270)	(2,952)
Accounts payable	1,836	3,178
Accrued liabilities	(2,893)	2,759
Operating lease liabilities	(406)	(315)
Net cash used in operating activities	(47,670)	(48,643)
Investing activities		
Purchase of debt securities available-for-sale	(92,480)	(96,267)
Proceeds from maturities of debt securities available-for-sale	74,767	45,271
Purchase of property and equipment	(107)	(94)
Net cash used in investing activities	(17,820)	(51,090)
Financing activities		
Issuance of common stock upon offering at-the-market, net of issuance costs	77,404	—
Proceeds from issuance of common stock upon stock option exercises	520	506
Proceeds from issuance of common stock upon ESPP purchase	186	130
Proceeds from issuance of common stock upon public offering, net of issuance costs	—	53,194
Proceeds from borrowings in connection with term loan, net of issuance costs	—	6,627
Repayment of accrued exit fee and second amendment fee	—	(913)
Repayment of term loan	—	(1,667)
Net cash provided by financing activities	78,110	57,877
Net increase (decrease) in cash and cash equivalents	12,620	(41,856)
Cash and cash equivalents at beginning of period	39,373	61,262
Cash and cash equivalents at end of period	\$ 51,993	\$ 19,406
Supplemental disclosure of cash flow information:		
Interest paid	\$ 2,089	\$ 1,944

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Eiger BioPharmaceuticals, Inc.
Notes to the Condensed Consolidated Financial Statements
(Unaudited)

1. Description of Business

Eiger BioPharmaceuticals, Inc. (the Company or Eiger) was incorporated in the State of Delaware on November 6, 2008. Eiger is a late-stage biopharmaceutical company focused on the development and commercialization of well-characterized drugs for life-threatening, rare and ultra-rare diseases with high unmet medical needs. Eiger has reported positive proof-of-concept clinical results across five programs: lonafarnib monotherapy in Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies, lonafarnib boosted with ritonavir in Hepatitis Delta Virus (HDV), peginterferon lambda (lambda) in HDV, and avexitide in both post-bariatric hypoglycemia (PBH) and congenital hyperinsulinism (CHI), all with first-in-class drugs, now under review for regulatory approvals, in Phase 3 clinical development, or advancing into Phase 3 development.

Eiger's lead clinical program is in Phase 3, developing lonafarnib, a first-in-class prenylation inhibitor, boosted with ritonavir, for the treatment of HDV infection, the most severe form of human viral hepatitis. The Company is also advancing lonafarnib monotherapy for treatment of Progeria and Progeroid Laminopathies, with a New Drug Application (NDA) and Marketing Authorization Application (MAA) under review by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA), respectively. Progeria and Progeroid Laminopathies are ultra-rare and rapidly fatal genetic conditions of accelerated aging in children. Lambda is the Company's second program treating HDV and is Phase 3 ready. Lambda is a well-characterized, late-stage, first-in-class, type III interferon. The Company is also developing avexitide, a well-characterized peptide, as a treatment for PBH, a debilitating and potentially life-threatening condition for which there is currently no approved therapy, and as a treatment CHI, an ultra-rare pediatric metabolic disorder.

The Company's principal operations are based in Palo Alto, California and it operates in one segment.

Liquidity

As of September 30, 2020, the Company had \$125.3 million of cash, cash equivalents and investments, comprised of \$52.0 million of cash and cash equivalents and \$73.3 million of debt securities available-for-sale. The Company had an accumulated deficit of \$287.7 million and negative cash flows from operating activities as of September 30, 2020. As we continue to incur losses, our transition to profitability will depend on the successful development, approval, and commercialization of product candidates and on the achievement of sufficient revenues to support our cost structure. We may never achieve profitability, and unless and until we do, we will need to continue to raise additional capital.

Management believes that the currently available resources will be sufficient to fund its operations for at least the next 12 months following the issuance date of these unaudited condensed consolidated financial statements. However, if our anticipated operating results are not achieved in future periods, we believe that planned expenditures may need to be reduced or we would be required to raise funding in order to fund our operations. Additionally, our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and the recent disruptions to, and volatility in, the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic.

2. Summary of Significant Accounting Policies

Basis of Presentation

The unaudited condensed consolidated financial statements include the accounts of Eiger BioPharmaceuticals, Inc. and its wholly owned subsidiaries, EBPI Merger Inc., EB Pharma LLC, Eiger BioPharmaceuticals Europe Limited, and EigerBio Europe Limited have been prepared in accordance with accounting principles generally accepted in the United States of America, (U.S. GAAP) and following the requirements of the Securities and Exchange Commission (the SEC) for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by U.S. GAAP can be condensed or omitted. These financial statements have been prepared on the same basis as the Company's annual financial statements and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments, which are necessary for a fair statement of the Company's financial information. These interim results are not necessarily indicative of the results to be expected for the nine months ended September 30, 2020 or for any other interim period or for any other future year. The balance sheet as of December 31, 2019, has been derived from audited consolidated financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements. All intercompany balances and transactions have been eliminated in consolidation.

The accompanying unaudited condensed consolidated financial statements and related financial information should be read in conjunction with the audited consolidated financial statements and the related notes thereto contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2019, filed with the Securities and Exchange Commission on March 13, 2020.

Use of Estimates

The preparation of unaudited condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. On an ongoing basis, the Company evaluates its estimates, including those related to clinical trial accrued liabilities, stock-based compensation, operating lease liabilities and income taxes. The Company bases its estimates on historical experience and on various other market-specific and relevant assumptions that the Company believes to be reasonable under the circumstances. Actual results could differ from those estimates.

Debt Securities

Short-term securities consist of debt securities classified as available-for-sale and have maturities greater than 90 days, but less than 365 days from the date of acquisition. All short-term securities are carried at fair value based upon quoted market prices. Unrealized gains and losses on available-for-sale securities are excluded from earnings and are reported as a component of accumulated other comprehensive income (loss). The cost of available-for-sale securities sold is based on the specific-identification method. Realized gains and losses on the sale of debt securities are determined using the specific-identification method and recorded in other expense, net on the accompanying unaudited condensed consolidated statements of operations.

Accrued Research and Development Costs

The Company accrues for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical and clinical studies, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced and includes these costs in accrued liabilities in the unaudited condensed consolidated balance sheets and within research and development expense in the unaudited condensed consolidated statements of operations. The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its third-party service providers. The Company makes judgments and estimates in determining the accrued liabilities balance in each reporting period. As actual costs become known, the Company adjusts its accrued liabilities.

Leases

The Company has a real estate lease for its office space in Palo Alto, California. The Company determines the initial classification and measurement of its right-of-use assets (ROU assets) and lease liabilities at the lease commencement date and thereafter if modified. The lease term includes any renewal options and termination options that the Company is reasonably assured to exercise. The present value of lease payments is determined by using the interest rate implicit in the lease, if that rate is readily determinable; otherwise, the Company uses its incremental borrowing rate. The incremental borrowing rate is determined by using the rate of interest that the Company would pay to borrow on a collateralized basis an amount equal to the lease payments for a similar term and in a similar economic environment.

Rent expense for operating leases is recognized on a straight-line basis, unless the operating lease ROU assets have been impaired, over the reasonably assured lease term based on the total lease payments and is included in operating expenses in the unaudited condensed consolidated statements of operations. For operating leases that reflect impairment, the Company will recognize the amortization of the operating lease ROU assets on a straight-line basis over the remaining lease term with rent expense still included in general and administrative expenses in the unaudited condensed consolidated statements of operations.

The Company elected the practical expedient to not separate lease and non-lease components. The Company's non-lease components are primarily related to property maintenance and insurance, which varies based on future outcomes, and thus is recognized in general and administrative expenses when incurred.

Net Loss per Share

Basic net loss per share of common stock is calculated by dividing the net loss by the weighted average number of shares of common stock outstanding during the period, without consideration for potentially dilutive securities. Since the Company was in a loss position for all periods presented, diluted net loss per share is the same as basic net loss per share for all periods as the inclusion of all potential common shares outstanding would have been anti-dilutive.

The following table sets forth the outstanding potentially dilutive securities which have been excluded in the calculation of diluted net loss per share because including such securities would be anti-dilutive (in common stock equivalent shares):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Options to purchase common stock	3,774,680	2,862,520	3,774,680	2,862,520
Restricted stock units (unvested)	37,500	—	37,500	—
Total	3,812,180	2,862,520	3,812,180	2,862,520

Recently Adopted Accounting Pronouncements

In August 2018, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2018-13, *Fair Value Measurement (Topic 820)*. The standard eliminates, modifies and adds disclosure requirements for fair value measurements. The pronouncement is effective for fiscal years, and for interim periods within those fiscal years, beginning after December 15, 2019, with early adoption permitted. The Company adopted this guidance on January 1, 2020. The adoption did not have a material impact on the Company's unaudited condensed consolidated financial statements.

In August 2018, the FASB issued ASU No. 2018-15, *Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That is a Service Contract (Subtopic 350-40)*. The standard adds certain disclosure requirements related to implementation costs incurred for internal-use software and cloud computing arrangements. The standard 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 (and hosting arrangements that include an internal-use software license). The pronouncement is effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years, with early adoption permitted. The amendments in ASU No. 2018-15 should be applied either using a retrospective or prospective approach. The Company adopted this guidance on July 1, 2020 using the prospective approach. The adoption did not have a material impact on the Company's condensed consolidated financial statements.

Accounting Pronouncements Not Yet Adopted

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326)*. The standard changes how entities will measure credit losses for most financial assets and certain other instruments that are not measured at fair value through net income. Financial assets measured at amortized cost will be presented at the net amount expected to be collected by using an allowance for credit losses. In April 2019, the FASB issued ASU No. 2019-04, *Codification Improvements to Topic 326, Financial Instruments – Credit Losses, Topic 815, Derivatives and Hedging, and Topic 825, Financial Instruments*, which clarifies and corrects certain unintended applications of the guidance contained in each of the amended Topics. Additionally, in May 2019, the FASB issued ASU No. 2019-05, *Financial Instruments – Credit Losses (Topic 326)*, which provides an option to irrevocably elect to measure certain individual financial assets at fair value instead of amortized cost. In November 2019, the FASB issued ASU No. 2019-10, *Financial Instruments – Credit Losses (Topic 326), Derivatives and Hedging (Topic 815), and Leases (Topic 842)*, which defers the effective date for ASU No. 2016-13 for smaller reporting companies to fiscal years beginning after December 15, 2022, including interim periods within those fiscal years. The Company is evaluating the impact of the guidance on its consolidated financial statements.

In March 2020, the FASB issued ASU No. 2020-4, *Reference Rate Reform (Topic 848)*. The standard provides optional expedients for facilitating the effects of the reference rate reform on financial reporting. For the Company, there are two applicable optional expedients for contract modifications permitted for contracts that are modified because of the reference rate reform and meet the scope guidance. The modifications of contracts within the scope of ASC Topic 470 should be accounted for prospectively adjusting the effective interest rate. The modifications of contracts within the scope of ASC Topic 842 should be accounted for as a continuation of the existing contracts with no reassessments of the lease classification and the discount rate or remeasurements of lease payments that otherwise would be required under ASC Topic 842 for modifications not accounted for as separate contracts. The pronouncement is effective for all entities as of March 12, 2020 through December 31, 2022. The Company plans to adopt upon the occurrence of such contract modification, but not later than December 31, 2022. The Company engaged in early-stage discussions with its lender and will assess the impact of the adoption once the contract is modified.

3. Fair Value Measurements

Fair value accounting is applied for all financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis (at least annually). As of September 30, 2020 and December 31, 2019, the carrying amount of prepaid expenses and other current assets, accounts payable and accrued liabilities approximated their estimate fair value due to their relatively short maturities. Management believes the terms of its long-term debt reflect current market conditions for an instrument with similar terms and maturity, therefore the carrying value of the Company's debt approximated its fair value.

Assets and liabilities recorded at fair value on a recurring basis in the unaudited condensed consolidated balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1: Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;

Level 2: Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and

Level 3: Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.

The Company's money market funds are classified as Level 1 because they are valued using quoted market prices. The Company's debt securities consist of available-for-sale securities and are classified as Level 2 because their value is based on valuations using significant inputs derived from or corroborated by observable market data. There were no assets or liabilities classified as Level 3 as of September 30, 2020 and December 31, 2019.

There were no transfers into or out of Level 3 of the fair value hierarchy during the periods presented.

The following tables present the fair value hierarchy for assets and liabilities measured at fair value (in thousands):

	September 30, 2020			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	\$ 48,190	\$ —	\$ —	\$ 48,190
Corporate debt securities	—	18,868	—	18,868
Commercial paper	—	5,492	—	5,492
U.S. treasury bills	—	48,981	—	48,981
Total	<u>\$ 48,190</u>	<u>\$ 73,341</u>	<u>\$ —</u>	<u>\$ 121,531</u>

	December 31, 2019			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	\$ 35,854	\$ —	\$ —	\$ 35,854
Corporate debt securities	—	16,644	—	16,644
Commercial paper	—	7,457	—	7,457
U.S. government Bonds	—	31,520	—	31,520
Total	<u>\$ 35,854</u>	<u>\$ 55,621</u>	<u>\$ —</u>	<u>\$ 91,475</u>

There were no financial liabilities as of September 30, 2020 and December 31, 2019.

The following tables summarize the estimated value of the Company's cash equivalents and debt securities and the gross unrealized holding gains and losses (in thousands):

	September 30, 2020			
	Amortized cost	Unrealized gain	Unrealized loss	Estimated Fair Value
Cash equivalents:				
Money market funds	\$ 48,190	\$ —	\$ —	\$ 48,190
Total cash equivalents	\$ 48,190	\$ —	\$ —	\$ 48,190
Debt securities:				
Corporate debt securities	\$ 18,855	\$ 18	\$ (5)	\$ 18,868
Commercial paper	5,492	—	—	5,492
U.S. treasury bills	48,978	3	—	48,981
Total debt securities	\$ 73,325	\$ 21	\$ (5)	\$ 73,341

	December 31, 2019			
	Amortized cost	Unrealized gain	Unrealized loss	Estimated Fair Value
Cash equivalents:				
Money market funds	\$ 35,854	\$ —	\$ —	\$ 35,854
Total cash equivalents	\$ 35,854	\$ —	\$ —	\$ 35,854
Debt securities:				
Corporate debt securities	\$ 16,633	\$ 11	\$ —	\$ 16,644
Commercial paper	7,457	—	—	7,457
U.S. government bonds	31,489	31	—	31,520
Total debt securities	\$ 55,579	\$ 42	\$ —	\$ 55,621

As of September 30, 2020 and December 31, 2019, the contractual maturity of the available-for-sale debt securities is less than one year. The Company periodically reviews the available-for-sale investments for other-than-temporary impairment loss. The Company considers factors such as the duration, severity and the reason for the decline in value, the potential recovery period and its intent to sell. For debt securities, it also considers whether (i) it is more likely than not that the Company will be required to sell the debt securities before recovery of their amortized cost basis, and (ii) the amortized cost basis cannot be recovered as a result of credit losses. During the nine months ended September 30, 2020, the Company did not recognize any other-than-temporary impairment losses. All debt securities with unrealized losses have been in a loss position for less than 12 months.

4. Balance Sheet Components

Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following (in thousands):

	September 30, 2020	December 31, 2019
Prepaid contract manufacturing costs	\$ 6,033	\$ 1,374
Prepaid research costs	1,995	1,627
Prepaid insurance	777	421
Other	803	1,968
Total prepaid expenses and other current assets	\$ 9,608	\$ 5,390

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	September 30, 2020	December 31, 2019
Contract research costs	\$ 3,309	\$ 5,288
Compensation and related benefits	2,135	1,707
Contract manufacturing costs	956	2,510
Other	662	496
Total accrued liabilities	<u>\$ 7,062</u>	<u>\$ 10,001</u>

5. Product Development Agreement

Product Development Agreement

On August 11, 2018, the Company entered into a Product Development Agreement and a First Project Agreement (the Product Agreements), pursuant to which the Company will receive development program support services for its HDV program. The services are to be provided from July 1, 2018 through the completion of the Phase 3 Clinical Study Reports and the subsequent new drug application (NDA) filing. As consideration, the Company has committed to pay fees of approximately \$10.0 million in cash and stock over four years, including approximately \$0.8 million for expert consultant fees and pass through costs to vendors, plus certain incentive-based regulatory milestone fees up to \$1.0 million. As part of the aggregate payment, the Company issued 115,526 shares of common stock subject to quarterly vesting requirements related to successful performance of the services and achievement of budget timeline set forth in the Product Agreements. The Product Agreements can be terminated by either party due to material breach or by the Company without cause with 90 days prior written notice. For the three and nine months ended September 30, 2020, the Company recognized research and development expense of \$0.1 million and \$0.2 million, respectively, related to the shares issued under the Product Agreements. Additionally, as of September 30, 2020, the total unrecognized compensation expense related to unvested restricted shares was \$0.3 million, which the Company expects to recognize over an estimated weighted-average period of 1.5 years.

6. Debt

In December 2016, the Company entered into an aggregate \$25.0 million loan with Oxford Finance LLC (the Oxford Loan). The loan matures on July 1, 2021. The Company borrowed \$15.0 million in December 2016 (Tranche A). In May 2018, the Company entered into an amendment to the Oxford Loan and borrowed \$5.0 million (Tranche B). On August 3, 2018, the Company borrowed the remaining \$5.0 million (Tranche C) under the Oxford Loan.

The Oxford Loan bears interest at a floating rate per annum equal to the greater of either the 30-day U.S. Dollar LIBOR reported in the Wall Street Journal plus 6.41% or 6.95%. The interest only period for borrowed funds was until February 1, 2019, followed by 30 equal monthly payments of principal plus accrued interest. At the time of final payment, the Company is required to pay an exit fee of 7.5% of the original principal balance of borrowed funds, or \$1.9 million. In addition, at the time of final payment of Tranche B, the Company is required to pay an additional exit fee of \$0.1 million. The Company recorded as a liability and debt discount the exit fee at the origination of the term loan. In addition, the Company incurred loan origination fees and debt issuance costs of \$0.4 million which were recorded as a direct deduction from the carrying amount of the related debt liability as a debt discount.

On March 5, 2019, the Company entered into the third amendment to the Oxford Loan (the Amended Oxford Loan) to refinance the Oxford Loan. The Amended Oxford Loan increased the aggregate amount available to be borrowed to \$35.0 million and extended the maturity date to March 1, 2024. On March 5, 2019, prior to entering into the Amended Oxford Loan, the outstanding balance of the Oxford Loan was \$23.3 million. At the time of entering into the Amended Oxford Loan, the Company borrowed an additional \$6.7 million in principal under the Amended Oxford Loan, which increased the total amount borrowed to \$30.0 million (Amended Tranche A). The remaining \$5.0 million (Amended Tranche B) is available to the Company provided that certain milestones are achieved by February 2021. The Company does not currently expect to draw down Amended Tranche B.

The Amended Oxford Loan bears interest at a floating rate per annum equal to the greater of either the 30-day U.S. Dollar LIBOR reported in the Wall Street Journal plus 6.64% or 9.15%. The Amended Oxford Loan has an interest only period until April 1, 2021, followed by 36 equal monthly payments of principal and interest. Upon the receipt of Amended Tranche B, the interest only period for borrowed funds will be extended by one year until April 1, 2022, followed by 24 equal monthly payments of principal plus accrued interest. At the time of final payment, the Company is required to pay an exit fee of 7.5% of the original principal balance of borrowed funds, or \$2.3 million. In addition, the Company is required to pay an additional exit fee of \$1.0 million. The Company recorded as a liability and debt discount the exit fee for the Amended Oxford Loan. At the date of the Amended Oxford Loan, the Company paid \$0.9 million for the accrued portion of the Oxford Loan exit fee and the Tranche B additional exit fee. The loan discount balance at the time of the Amended Oxford Loan was \$0.2 million, which is being amortized over the remaining term of the Amended Oxford Loan.

The Company is also required to pay a 5.0% success fee of the total amount drawn under the Amended Oxford Loan within 30 days following the FDA's approval of the Company's first product, excluding lonafarnib in Progeria and Progeroid Laminopathies. This fee is enforceable within 10 years from the funding of Amended Tranche A. The Company determined that the success fee met the scope exemption from derivative accounting and should be accounted for under the guidance for contingencies. Accordingly, the Company will record a liability for the success fee upon receipt of approval from the FDA. The Amended Oxford Loan includes contingent interest features and mandatory prepayment features upon an event of default that meet the definition of a derivative but were not bifurcated from the debt instrument as their fair value was deemed to be insignificant. In connection with the execution of the Oxford Loan, the Company agreed to certain customary representations and warranties.

The refinancing of the term loan did not have a material impact on terms, conditions, representations, warranties, covenants or agreements set forth in the Oxford Loan. The loan is secured by the perfected first priority liens on the Company's assets, including a commitment by the Company to not allow any liens to be placed upon the Company's intellectual property. The loan includes customary events of default, including failure to pay amounts due, breaches of covenants and warranties, material adverse effect events, certain cross defaults and judgments, and insolvency. If the Company is unable to comply with these covenants or if the Company defaults on any portion of the outstanding borrowings, the lenders can also impose a 5.0% penalty, restrict access to additional borrowings under the loan and security agreement, and accelerate the maturity of the debt.

The Company is permitted to make voluntary prepayments of the Amended Oxford Loan with a prepayment fee, calculated as of the loan origination date, equal to (i) 2.0% of the loan prepaid during the first 12 months and (ii) 1.0% of the loan prepaid in months 13-24. The Company is required to make mandatory prepayments of the outstanding loan upon the acceleration by lender following the occurrence of an event of default, along with a payment of the final payment, the prepayment fee and any other obligations that are due and payable at the time of prepayment.

The Company accounts for the amortization of the debt discount utilizing the effective interest method. Long-term debt and unamortized discount balances are as follows (in thousands):

	September 30, 2020	December 31, 2019
Face value of long-term debt	\$ 30,000	\$ 30,000
Exit fee	3,277	3,277
Unamortized debt discount associated with exit fee, debt issuance costs and loan origination fees	(2,294)	(2,887)
Total long-term debt	30,983	30,390
Less: current portion of long-term debt, net	5,783	—
Long-term debt, net	<u>\$ 25,200</u>	<u>\$ 30,390</u>

7. Stock-Based Compensation

The following table summarizes stock option activity under the Company's stock-based compensation plan during the nine months ended September 30, 2020 (in thousands, except option and share data):

	Shares Available for Grant	Number of Options	Weighted- Average Exercise Price	Weighted- Average Remaining Contractual Life (in Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2019	823,598	2,767,617	\$ 12.14	7.94	\$ 8,218
Additional options authorized	1,226,169	—			
Granted	(1,149,750)	1,149,750	\$ 7.43		
Restricted stock units granted	(37,500)	—			
Exercised	—	(61,700)	\$ 8.41		
Canceled and forfeited	80,987	(80,987)	\$ 11.18		
Outstanding as of September 30, 2020	<u>943,504</u>	<u>3,774,680</u>	<u>\$ 10.78</u>	<u>7.86</u>	<u>\$ 2,921</u>
Vested and exercisable as of September 30, 2020		<u>1,856,770</u>	<u>\$ 11.69</u>	<u>6.87</u>	<u>\$ 1,245</u>

During the three and nine months ended September 30, 2020, the Company granted stock options for the purchase of 85,000 and 1,149,750 shares of the Company's common stock with a weighted-average grant date fair value of \$7.18 and \$4.78 per share, respectively. During the three and nine months ended September 30, 2019, the Company granted stock options for the purchase of 217,000 and 1,079,750 shares of the Company's common stock with a weighted-average grant date fair value of \$7.18 and \$9.01 per share, respectively.

The Company records stock-based compensation of stock options granted by estimating the fair value of stock-based awards using the Black-Scholes option pricing model and amortizes the fair value of the stock-based awards granted over the applicable vesting period of the awards on a straight-line basis. The fair value of stock options was estimated using the following weighted-average assumptions:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Expected term (in years)	6.08	6.02-6.08	5.27-6.08	5.27-6.08
Contractual term (in years)	—	—	10.00	10.00
Volatility	74.00%	74.19%-75.70%	73.00%-77.00%	74.19%-83.19%
Risk free interest rate	0.39%	1.42%-1.90%	0.39%-1.37%	1.42%-2.57%
Dividend yield	—	—	—	—

Stock-Based Compensation Expense

Total stock-based compensation expense recognized was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	\$ 367	\$ 559	\$ 1,154	\$ 1,366
General and administrative	1,078	1,090	3,382	2,965
Total	\$ 1,445	\$ 1,649	\$ 4,536	\$ 4,331

As of September 30, 2020, the total unrecognized compensation expense related to unvested options was \$12.1 million, which the Company expects to recognize over an estimated weighted average period of 2.7 years.

Restricted Stock Units

In the first quarter of 2020, the Company revised its non-employee director compensation policy to approve the granting of restricted stock units (RSUs) in accordance with the Restated 2013 Equity Incentive Plan (the Restated 2013 Plan). Each eligible director who has served for less than six months during the prior calendar year and continues to serve as a non-employee member of the board is granted RSUs, which are pro-rated for the period served during the prior calendar year.

The RSUs granted to non-employee directors will vest on the one-year anniversary of the grant date, subject to the eligible director's continuous services through the vesting date, and will vest in full upon a change in control, as defined under the Restated 2013 Plan. For RSU's granted to employees during the nine months ended September 30, 2020, the vesting will begin on the one-year anniversary through the two-year anniversary of the grant date. The fair value of all RSUs are measured at the grant date based on the closing market price of the Company's common stock and is recognized as stock-based compensation expense on a straight-line basis over the vesting period.

There were no RSUs granted during the three months ended September 30, 2020. For the nine months ended September 30, 2020, 37,500 RSUs with a weighted-average grant date fair value of \$6.95 per share. The Company recognized \$0.1 million and \$0.1 million in stock-based compensation expense for the three and nine months ended September 30, 2020, respectively, which is included in general and administrative expenses. As of September 30, 2020, the total unrecognized compensation expense related to unvested RSUs was \$0.1 million, which the Company expects to recognize over an estimated weighted-average period of 0.6 years.

8. Income Taxes

The Company did not record tax expense for the three and nine months ended September 30, 2020 and 2019 due to the Company's loss position and full valuation allowance.

9. Commitments and Contingencies

Lease Agreements

In October 2017, the Company entered into a non-cancelable operating facility lease agreement for 8,029 square feet of office space located at 2155 Park Blvd. in Palo Alto, California 94306. The lease commenced on March 1, 2018 and expires in February 2023. The lease has a three-year renewal option prior to expiration; however, the Company is not reasonably assured to exercise this option. The lease includes rent escalation clauses throughout the lease term. In October 2017, the Company provided a security deposit of \$0.3 million. The Company also has three additional operating leases that are included in its lease accounting but are not considered significant for disclosure.

The maturity of the Company's operating lease liabilities were as follows (in thousands):

Undiscounted lease payments	September 30, 2020
Remaining in 2020	\$ 160
2021	673
2022	662
2023	113
Total undiscounted payments	1,608
Less: imputed interest	160
Present value of future lease payments	1,448
Less: current portion of operating lease liabilities	565
Operating lease liabilities	\$ 883

Rent expense recognized for the Company's operating leases was \$0.2 million and \$0.1 million for the three months ended September 30, 2020 and 2019, respectively, and \$0.5 million and \$0.4 million for the nine months ended September 30, 2020 and 2019, respectively. Under the terms of the lease agreements, the Company is also responsible for certain variable lease payments that are not included in the measurement of the lease liability. Variable lease payments for the operating leases were \$23,000 and \$21,400 for the three months ended September 30, 2020 and 2019, respectively, and \$0.1 million and \$0.1 million for the nine months ended September 30, 2020 and 2019.

The operating cash outflows for the operating lease liabilities were \$0.5 million and \$0.5 million for the nine months ended September 30, 2020 and 2019, respectively. As of September 30, 2020 and December 31, 2019, the weighted-average remaining lease terms were 2.4 years and 3.1 years, and weighted-average discount rates were 9.15% and 9.15%, respectively.

10. Subsequent Events

As of October 27, 2020, the Company completed the sale of common stock subject to its ATM financing facility with Jefferies LLC under the effective shelf statement filed on August 6, 2020, resulting in the total offering of 615,654 shares of common stock and net proceeds to the Company of \$6.1 million, after deducting commissions.

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

You should read the following discussion and analysis of Eiger's financial condition and results of operations together with our unaudited condensed consolidated financial statements and related notes thereto included in Part I, Item 1 of this Quarterly Report on Form 10-Q, and our consolidated financial statements and related notes thereto for the year ended December 31, 2019, included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on March 13, 2020. This discussion and other parts of this report contain forward-looking statements that involve risks and uncertainties, such as our plans, objectives, expectations, intentions and beliefs. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section entitled "Risk Factors" included elsewhere in this report.

Forward-Looking Statements

This Quarterly Report contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. In some cases, forward-looking statements are identified by words such as "believe," "will," "may," "estimate," "continue," "anticipate," "intend," "should," "plan," "expect," "predict," "could," "potentially" or the negative of these terms or similar expressions. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to, among other things, our future plans, objectives, expectations, intentions, the potential for our programs, the timing of our clinical trials and financial performance and the assumptions that underlie these statements. These forward-looking statements are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements. Factors that might cause such a difference include, but are not limited to, those discussed in this Quarterly Report on Form 10-Q in Part II, Item 1A — "Risk Factors," and elsewhere in this Quarterly Report on Form 10-Q. Forward-looking statements are based on our management's beliefs and assumptions and on information currently available to our management. These statements, like all statements in this Quarterly Report on Form 10-Q, speak only as of their date, and we undertake no obligation to update or revise these statements in light of future developments. We caution investors that our business and financial performance are subject to substantial risks and uncertainties.

Overview

We are a late-stage biopharmaceutical company focused on the development and commercialization of well-characterized drugs for serious, rare and ultra-rare diseases with high unmet medical needs. We have reported positive proof-of-concept clinical results across all our programs, now under review for regulatory approvals, in Phase 3 clinical development, or advancing into Phase 3 development. Four of our five programs have Breakthrough Therapy designation.

Our programs have several aspects in common: the disease targets represent conditions of high unmet medical need; the therapeutic approaches are supported by an understanding of disease biology and mechanism as elucidated by our academic research relationships; prior clinical experience with the product candidates guides an understanding of safety; and the development paths leverage the experience and capabilities of our experienced, commercially-focused management team.

Our lead clinical program is in Phase 3, developing lonafarnib, a first-in-class prenylation inhibitor, boosted with ritonavir, for the treatment of Hepatitis Delta Virus (HDV) infection, the most severe form of human viral hepatitis. The pivotal Phase 3 D-LIVR study (n=400) is ongoing and enrolling patients. The study spans over twenty countries and one hundred sites and has potential to generate data for two lonafarnib-based ritonavir-boosted regimens for approval. An all-oral arm of lonafarnib boosted with ritonavir and a combination arm of lonafarnib boosted with ritonavir combined with pegylated interferon-alfa-2a will each be compared to placebo. We are also developing lonafarnib monotherapy for treatment of Progeria and Progeroid Laminopathies. In March 2020, we submitted a New Drug Application (NDA) to the Food & Drug Administration (FDA) and a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA). The NDA was accepted for filing by the FDA in May 2020 with priority review, and the MAA has been validated by the EMA and is under a standard review timeline. Progeria and Progeroid Laminopathies are ultra-rare and rapidly fatal genetic conditions of accelerated aging in children.

Peginterferon lambda (lambda) is our second program treating HDV and is Phase 3 ready. We have agreement from FDA and EMA on a single pivotal phase 3 study design and endpoints. Lambda is a well-characterized, late-stage, first in class, type III interferon. We previously reported Phase 2 LIMIT (lambda monotherapy) study results (n=33) that demonstrated a 36% durable virologic response (DVR), or below the limit of quantification (BLQ), at 24 weeks post-treatment. In the Phase 2 LIFT study, HDV patients were treated with a combination of our two proprietary products, lambda and lonafarnib boosted by ritonavir. The end-of-treatment results reported in August 2020 showed that 58% of patients were HDV RNA undetectable or BLQ at Week 24, and 95% of patients achieved the primary end-point of >2 log decline in HDV RNA at Week 24. Adverse events were mostly mild to moderate.

We are also developing avexitide, a well-characterized peptide, as a treatment for post-bariatric hypoglycemia (PBH), a debilitating and potentially life-threatening condition for which there is currently no approved therapy. We have completed four clinical studies demonstrating proof of concept in 54 patients suffering from severe, refractory PBH, and have received FDA guidance on a single pivotal Phase 3 trial.

Avexitide has also demonstrated proof of concept for treatment of congenital hyperinsulinism (CHI), an ultra-rare pediatric metabolic genetic disorder.

We have no products approved for commercial sale and have not generated any revenue from product sales. We have never been profitable and have incurred operating losses in each year since inception, and we do not anticipate that we will achieve profitability in the near term. Our net losses were \$46.2 million and \$53.3 million for the nine months ended September 30, 2020 and 2019, respectively. As of September 30, 2020, we had an accumulated deficit of \$287.7 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We expect to incur significant expenses and increasing operating losses for at least the next several years as we initiate and continue the clinical development of, and seek regulatory approval for, and potentially commercialize our product candidates and add personnel necessary to operate as a public company with an advanced clinical candidate pipeline of products. In addition, we will incur costs for additional personnel and upgrades to our information technology systems as we transition from an emerging growth company. We expect that our operating losses will fluctuate significantly from quarter to quarter and year to year due to timing of clinical development programs and efforts to achieve regulatory approval.

Recent Developments

Phase 2 LIFT HDV Study of Peginterferon Lambda (Lambda) Combined with Lonafarnib End-of-Treatment Data

On August 31, 2020, we announced positive end-of-treatment data from the Phase 2 LIFT study. At Week 24 end of treatment, 25 of 26 patients (96%) achieved the Primary Endpoint of > 2 log decline in HDV RNA, 58% of patients were either Below Limit of Quantitation or Undetectable, and median HDV RNA decline was 3.2 log IU/mL (CI: 2.50-3.93, $p < 0.0001$). Combination therapy with lambda and Lonafarnib in chronic HDV patients appears to be well tolerated for up to six months in most patients. Adverse events were mostly mild to moderate and included GI-related side effects, weight loss, hyperbilirubinemia, and anemia. Per-protocol dose reductions occurred in three patients and discontinued in four patients, and were mostly due to known side effects related to peginterferon lambda.

Investigator Sponsored Studies of Peginterferon Lambda (Lambda) in COVID-19

On October 15, 2020, we announced positive results of the ILIAD Study (Interferon Lambda for Immediate Antiviral Therapy at Diagnosis in COVID-19), an investigator sponsored randomized trial of lambda in outpatients with mild to moderate COVID-19 conducted at Toronto General Hospital, University Health Network in Toronto, Canada. The main efficacy outcomes were viral load decline and the proportion of individuals with a negative nasopharyngeal swab for SARS-CoV-2 at Day 7. A total of 60 patients were randomized 1:1 to a single subcutaneous dose of lambda 180 mcg or normal saline placebo. Patients were followed for 14 days. The cutoff of 6 log copies/mL was prospectively chosen as this correlates with the threshold for infectivity and the likely need for treatment.

The SARS-CoV-2 RNA viral load decline from baseline was significantly greater in the lambda group than in the placebo group from Day 5 onwards. After controlling for baseline viral load, those treated with lambda were 4.1-fold (95% CI 1.2-16.7, $p = 0.029$) more likely to clear by Day 7 than those in the placebo arm. For those with baseline viral load > 6 log copies/mL, the proportion negative at Day 7 in the lambda group was 15 of 19 (79%) compared to 6 of 16 (38%) in the placebo group ($p = 0.013$). Across all patients, by Day 7, 24 of 30 patients (80%) in the lambda group were negative compared to 19 of 30 (63%) in the placebo arm ($p = 0.15$). Lambda was well-tolerated with few adverse events, which included minimal elevations of transaminases which self-resolved.

On September 28, 2020, we announced results of an investigator sponsored study of lambda in outpatients with mild and uncomplicated COVID-19 conducted at Stanford University. The primary endpoint was duration of viral shedding, determined by time to first of two consecutive negative tests for SARS-CoV-2 by qRT-PCR. The secondary endpoint was reducing duration of symptoms and hospitalization in patients with mild COVID-19. A total of 120 patients were randomized 1:1 to a single subcutaneous dose of lambda or normal saline placebo. Patients were followed for 28 days. No difference was demonstrated in duration of SARS-CoV-2 viral shedding and time to symptom resolution when compared with placebo. Median time to cessation of viral shedding in both groups was 7 days. Lambda was well-tolerated with few adverse events, which included elevated transaminases which self-resolved.

Update on Impact of COVID-19 Pandemic on Clinical Development Activities and Business Operations

We have taken proactive steps to ensure the safety of patients and the integrity of our HDV Phase 3 D-LIVR trial, which is expected to complete enrollment in 2021. We have adequate clinical drug product supply for the D-LIVR study and do not anticipate any interruption in availability of study drug to patients.

We do not anticipate that the FDA reviews of our NDA for lonafarnib for Progeria and Progeroid Laminopathies will be impacted by COVID-19. We also do not anticipate any impact to our planned U.S. commercial launch, including availability of commercial drug supply. We previously announced that the EMA review of our MAA will follow a standard review timeline.

We have put into place remote operations and new policies, which are in-line with local, state and federal guidelines, to maintain the safety and well-being of our employees, while working to maintain business continuity as this unprecedented global situation continues to evolve. We continue to monitor the situation closely, including its potential effect on our clinical development plans and timelines.

Financial Operations Overview

Research and Development Expenses

Research and development expenses represent costs incurred to conduct research and development, such as the development of our product candidates. We recognize all research and development costs as they are incurred. Research and development expenses consist primarily of the following:

- expenses incurred under agreements with consultants, contract research organizations and clinical trial sites that conduct research and development activities on our behalf;
- laboratory and vendor expenses related to the execution of clinical trials;
- contract manufacturing expenses, primarily for the production of clinical trial supplies;
- license fees associated with our license agreements; and
- internal costs that are associated with activities performed by our research and development organization and generally benefit multiple programs. These costs are not separately allocated by product candidate. Unallocated internal research and development costs consist primarily of:
 - personnel costs, which include salaries, benefits and stock-based compensation expense;
 - allocated facilities and other expenses, which include expenses for rent and maintenance of facilities and depreciation expense; and
 - regulatory expenses and technology license fees related to development activities.

The largest component of our operating expenses has historically been the investment in clinical trials, including contract manufacturing arrangements, clinical trial material related costs and other research and development activities. However, we do not allocate internal research and development costs, such as salaries, benefits, stock-based compensation expense and indirect costs to product candidates on a program-specific basis. The following table shows our research and development expenses for the three and nine months ended September 30, 2020 and 2019 (in thousands):

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
Product candidates:				
Lonafarnib	\$ 6,082	\$ 9,654	\$ 18,997	\$ 29,621
Lambda	1,061	895	2,238	1,437
Avexitide	431	1,349	1,632	2,714
Internal research and development costs	2,236	2,161	6,178	6,091
Total research and development expense	<u>\$ 9,810</u>	<u>\$ 14,059</u>	<u>\$ 29,045</u>	<u>\$ 39,863</u>

We expect research and development expenses will increase in the future as we advance our product candidates into and through later stage clinical trials and pursue regulatory approvals, which will require a significant investment in regulatory support and contract manufacturing and clinical trial material related costs. In addition, we continue to evaluate opportunities to acquire or in-license other product candidates and technologies, which may result in higher research and development expenses due to license fees and/or milestone payments.

The process of conducting clinical trials necessary to obtain regulatory approval is costly and time consuming. We may never succeed in timely developing and achieving regulatory approval for our product candidates. The probability of success of our product candidates may be affected by numerous factors, including clinical data, competition, intellectual property rights, manufacturing capability and commercial viability. As a result, we are unable to determine the duration and completion costs of our development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates. The COVID-19 pandemic presents additional risks and uncertainties associated with developing drugs, including:

- delays in trial activities and patient enrollment or diversion of healthcare resources as a result of the evolving effects of the COVID-19 pandemic or otherwise;
- production shortages or other supply interruptions in clinical trial materials resulting from the evolving effects of the COVID-19 pandemic or otherwise;
- our ability to hire and retain key research and development personnel;
- the scope, rate of progress, results and expense of our ongoing, as well as any additional, clinical trials and other research and development activities; and
- the timing and receipt of any regulatory approvals.

General and Administrative Expenses

General and administrative expenses consist of personnel costs, allocated expenses and expenses for outside professional services, including legal, audit, accounting services, insurance costs and costs associated with being a public company. Personnel costs consist of salaries, benefits and stock-based compensation. Allocated expenses consist of facilities and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation expense and other supplies. Our expenses include costs related to compliance with the rules and regulations of the SEC and Nasdaq, insurance, investor relations, banking fees and other administrative expenses and professional services.

Interest Expense

Interest expense consists of interest and amortization of the debt discount related to the Oxford Loan.

Interest Income

Interest income consists of interest earned on our investments in debt securities and cash equivalents.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our unaudited condensed consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of these unaudited condensed consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses. On an ongoing basis, we evaluate these estimates and judgments. We base our estimates on historical experience and on various assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Accrued Research and Development Costs

We record accrued expenses for estimated costs of research and development activities conducted by external service providers, which include the conduct of clinical research and contract formulation and manufacturing activities. We record the estimated costs of development activities based upon the estimated amount of services provided but not yet invoiced and include these costs in accrued liabilities in the unaudited condensed consolidated balance sheet and within research and development expense in the unaudited condensed consolidated statements of operations. We record accrued expenses for these costs based on the estimated amount of work completed and in accordance with agreements established with these external service providers.

We estimate the amount of work completed through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and the agreed-upon fee to be paid for such services. We make judgments and estimates in determining the accrued balance in each reporting period. As actual costs become known, we adjust our accrued estimates.

Stock-Based Compensation

We recognize compensation costs related to stock options and restricted stock units based on the estimated fair value of the awards on the date of grant. We estimate the grant date fair value of stock options, and the resulting stock-based compensation expense, using the Black-Scholes option-pricing model. The grant date fair value of the stock-based awards is generally recognized on a straight-line basis over the requisite service period, which is generally the vesting period of the respective awards. We record forfeitures when they occur.

The Black-Scholes option-pricing model includes the following assumptions:

Expected Term. Our expected term represents the period that our stock-based awards are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term).

Expected Volatility. Since we have only been publicly traded for a short period and do not have adequate trading history for our common stock, the expected volatility was estimated based on the average volatility for comparable publicly traded biopharmaceutical companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on their similar size, stage in the life cycle, or area of specialty. Beginning in the third quarter of 2019, as we had been publicly traded for four and a half years, we began to layer in our historical volatility in the calculation of expected volatility.

Risk-Free Interest Rate. The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.

Expected Dividend. We have never paid dividends on our common stock and have no plans to pay dividends on our common stock. Therefore, we used an expected dividend yield of zero.

We use the contractual term to determine the non-employee awards' fair value at the grant date. The contractual term of options granted under the Plan is 10 years. Our Board of Directors determined the fair value of each share of underlying common stock based on the closing price of our common stock as reported by the Nasdaq Global Market on the date of grant.

We estimate the fair value of restricted stock units based on the closing market price of our common stock on the date of grant and the resulting stock-based compensation expense is recognized on a straight-line basis over the requisite service period, which is generally the vesting period of the awards.

Results of Operations

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

	Three Months Ended September 30,		\$	%
	2020	2019	Change	Change
Operating expenses:				
Research and development	\$ 9,810	\$ 14,059	\$ (4,249)	(30%)
General and administrative	5,027	4,247	780	18%
Total operating expenses	14,837	18,306	(3,469)	(19%)
Loss from operations	(14,837)	(18,306)	3,469	
Interest expense	(906)	(884)	(22)	*
Interest income	76	585	(509)	(87%)
Other expense, net	(13)	(11)	(2)	*
Net loss	\$ (15,680)	\$ (18,616)	\$ 2,936	(16%)

*Percentage not meaningful.

Research and development expenses

Research and development expenses decreased by \$4.3 million to \$9.8 million for the three months ended September 30, 2020, from \$14.1 million for the same period in 2019. The decrease was primarily due to a \$4.1 million decrease in contract manufacturing and clinical expenditures due to a decrease in program activities and a \$0.2 million decrease in compensation and personnel related expenses, including stock-based compensation expense.

General and administrative expenses

General and administrative expenses increased by \$0.8 million to \$5.0 million for the three months ended September 30, 2020, from \$4.2 million for the same period in 2019. The increase was primarily due to a \$0.5 million increase in outside legal, consulting, advisory and accounting services and a \$0.3 million increase in compensation and personnel related expenses.

Interest expense

Interest expense remained relatively flat for the three months ended September 30, 2020 and for the same period in 2019.

Interest income

Interest income decreased by \$0.5 million to \$0.1 million for the three months ended September 30, 2020 from \$0.6 million for the same period in 2019. The decrease was primarily due to a decrease in interest rates on money market funds.

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

	Nine Months Ended September 30,		\$ Change	% Change
	2020	2019		
Operating expenses:				
Research and development	\$ 29,045	\$ 39,863	\$ (10,818)	(27%)
General and administrative	15,141	12,529	2,612	21%
Total operating expenses	44,186	52,392	(8,206)	(16%)
Loss from operations	(44,186)	(52,392)	8,206	
Interest expense	(2,681)	(2,518)	(163)	6%
Interest income	629	1,598	(969)	(61%)
Other expense, net	(7)	(20)	13	*
Net loss	\$ (46,245)	\$ (53,332)	\$ 7,087	(13%)

*Percentage not meaningful.

Research and development expenses

Research and development expenses decreased by \$10.9 million to \$29.0 million for the nine months ended September 30, 2020, from \$39.9 million for the same period in 2019. The decrease was primarily due to a \$10.0 million decrease in contract manufacturing and clinical expenditures due to a decrease in program activities and a \$1.0 million decrease in regulatory expenses.

General and administrative expenses

General and administrative expenses increased by \$2.6 million to \$15.1 million for the nine months ended September 30, 2020, from \$12.5 million for the same period in 2019. The increase was primarily due to a \$1.4 million increase in compensation and personnel related expenses, including stock-based compensation expense due to an increase in headcount, and a \$1.1 million increase in outside legal, consulting, advisory and accounting services.

Interest expense

Interest expense increased by \$0.2 million to \$2.7 million for the nine months ended September 30, 2020 from \$2.5 million for the same period in 2019. Interest expense primarily increased due to the additional funds borrowed under the Oxford Loan in March 2019.

Interest income

Interest income decreased by \$1.0 million to \$0.6 million for the nine months ended September 30, 2020 from \$1.6 million for the same period in 2019. The decrease was primarily due to a decrease in interest rates on money market funds.

Liquidity and Capital Resources

Sources of Liquidity

As of September 30, 2020, we had \$125.3 million of cash, cash equivalents and investments, comprised of \$52.0 million of cash and cash equivalents and \$73.3 million of debt securities available-for-sale, and an accumulated deficit of \$287.7 million.

In December 2019, we filed a shelf registration statement on Form S-3 (File No. 333-235655) with the Securities and Exchange Commission, which permits the offering, issuance and sale by us of up to a maximum aggregate offering price of \$150.0 million of our common stock, preferred stock, debt securities and warrants. Up to a maximum of \$50.0 million of the maximum aggregate offering price of \$150.0 million may be issued and sold pursuant to an at-the-market (ATM) financing facility (2019 ATM Facility) under a sales agreement with Jefferies LLC (Jefferies).

During the nine months ended September 30, 2020, we completed ATM offerings under the 2019 ATM Facility and the new sales agreement (the 2020 ATM Facility) with Jefferies for a total of 7,305,313 shares resulting in \$77.6 million in net proceeds, after deducting commissions. From October 1, 2020 through October 27, 2020, we completed ATM offerings under the 2020 ATM Facility for a total of 615,654 shares and \$6.1 million in net proceeds, after deducting commissions. Following such issuances, as of October 31, 2020, \$13.8 million of common stock remains available to be sold under the 2020 ATM Facility, subject to certain conditions as specified in the Sales Agreement.

We believe that the currently available resources will be sufficient to fund our operations for at least the next 12 months following the issuance date of these unaudited condensed consolidated financial statements. However, if our anticipated operating results are not achieved in future periods, we believe that planned expenditures may need to be reduced or we would be required to raise funding in order to fund our operations. Additionally, our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and the recent disruptions to, and volatility in, the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic.

Our primary uses of cash are to fund operating expenses, including research and development expenditures and general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in outstanding accounts payable and accrued expenses.

Future Funding Requirements

We have not generated any revenue from product sales. We do not expect to generate any revenue from product sales unless and until we obtain regulatory approval for and commercialize any of our product candidates. At the same time, we expect our expenses to increase in connection with our ongoing development and manufacturing activities, particularly as we continue the research, development, manufacture and clinical trials of, and seek regulatory approval for our product candidates.

Our primary uses of capital are, and we expect will continue to be, funding research efforts and the development of our product candidates, compensation and related expenses, hiring additional staff, including clinical, scientific, operational, financial, and management personnel, and costs associated with operating as a public company. We expect to incur substantial expenditures in the foreseeable future for the development and potential commercialization of our product candidates.

We plan to continue to fund losses from operations and capital funding needs through future equity and/or debt financings, as well as potential additional collaborations or strategic partnerships with other companies. As a result of economic conditions, general global economic uncertainty, political change and other factors, including the ongoing COVID-19 pandemic, we do not know whether additional capital will be available when needed, or that, if available, we will be able to obtain additional capital on reasonable terms. If we are unable to raise additional capital due to the volatile global financial markets, general economic uncertainty or other factors, we may need to curtail planned development or commercialization activities. The sale of additional equity or convertible debt could result in additional dilution to our stockholders. The incurrence of indebtedness would result in debt service obligations and could result in operating and financing covenants that would restrict our operations. We can provide no assurance that financing will be available in the amounts we need or on terms acceptable to us, if at all. If we are not able to secure adequate additional funding we may be forced to delay, make reductions in spending, extend payment terms with suppliers, liquidate assets where possible, and/or suspend or curtail planned programs. Any of these actions could materially harm our business.

Cash Flows

The following table summarizes our cash flows for the periods indicated (in thousands):

	Nine Months Ended September 30,	
	2020	2019
Net cash used in operating activities	\$ (47,670)	\$ (48,643)
Net cash used in investing activities	(17,820)	(51,090)
Net cash provided by financing activities	78,110	57,877
Net increase (decrease) in cash and cash equivalents	\$ 12,620	\$ (41,856)

Cash flows from operating activities

Cash used in operating activities for the nine months ended September 30, 2020 was \$47.7 million, which primarily consisted of a net loss of \$46.2 million, partially offset by stock-based compensation expense of \$4.5 million, non-cash interest expense of \$0.6 million, amortization of operating lease right-of-use assets of \$0.4 million and common stock issued under Product Development Agreement of \$0.2 million. Additionally, cash used in operating activities reflected changes in net operating assets of \$7.2 million due to an increase of \$4.4 million in prepaid expenses and other current assets primarily due to the timing of payments, an increase of \$1.3 million in other assets primarily related to long term deposits with clinical research organizations, a decrease of \$1.1 million in accounts payable and accrued liabilities due to timing of payments and a decrease of \$0.4 million in operating lease liabilities.

Cash used in operating activities for the nine months ended September 30, 2019 was \$48.6 million, which primarily consisted of a net loss of \$53.3 million and amortization of the debt securities discount of \$0.4 million, partially offset by stock-based compensation expense of \$4.3 million, non-cash interest expense of \$0.5 million, and amortization of operating lease right-of-use assets of \$0.3 million. Additionally, cash used in operating activities reflected changes in net operating assets of \$0.3 million due to an increase of \$3.0 million in prepaid expenses and other current assets primarily due to the timing of payments, an increase of \$2.9 million in other assets primarily related to long term deposits with clinical research organizations, and a decrease of \$0.3 million in operating lease liabilities, which was partially offset by an increase of \$5.9 million in accounts payable and accrued liabilities due to the timing of payments.

Cash flows from investing activities

Cash used in investing activities was \$17.8 million for the nine months ended September 30, 2020, primarily consisting of \$92.5 million of purchases of debt securities, partially offset by \$74.8 million of proceeds from maturities of debt securities.

Cash used in investing activities was \$51.1 million for the nine months ended September 30, 2019, consisting of \$96.3 million of purchases of debt securities and \$0.1 million of purchases of property and equipment, partially offset by \$45.3 million of proceeds from maturities of debt securities.

Cash flows from financing activities

Cash provided by financing activities for the nine months ended September 30, 2020 primarily consisted of \$77.4 million of net proceeds from the issuance of common stock upon offering at-the-market and \$0.7 million of proceeds from the issuance of common stock upon stock option exercises and ESPP purchase.

Cash provided by financing activities for the nine months ended September 30, 2019 consisted of \$53.2 million of net proceeds from the issuance of common stock upon public offering in April 2019, \$6.6 million of net proceeds from additional borrowings in connection with the restructuring of the Oxford Loan and \$0.6 million of proceeds from the issuance of common stock upon stock option exercises and ESPP purchase, partially offset by \$2.6 million in payments of principal and fees associated with the Oxford Loan.

Contractual Obligations and Other Commitments

Our contractual obligations as of September 30, 2020 have not materially changed from what we presented in our Annual Report on Form 10-K for the year ended December 31, 2019 filed with the SEC on March 13, 2020.

Off-Balance Sheet Arrangements

During the periods presented, we did not have, nor do we currently have, any off-balance sheet arrangements as defined under the rules of the SEC and do not have any holdings in variable interest entities.

ITEM 3. Quantitative and Qualitative Disclosures about Market Risk

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934 and are not required to provide the information under this item.

ITEM 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We are responsible for maintaining disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Disclosure controls and procedures are controls and other procedures designed to ensure that the information required to be disclosed by us in the reports that 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. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and our principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

Based on our management's evaluation (with the participation of our principal executive officer and our principal financial officer) of our disclosure controls and procedures as required by Rule 13a-15 under the Exchange Act, our principal executive officer and our principal financial officer have concluded that our disclosure controls and procedures were effective to achieve their stated purpose as of September 30, 2020, the end of the period covered by this report.

Management's Report on Internal Control over Financial Reporting.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as such term is defined in Exchange Act Rule 13a-15(f). Internal control over financial reporting is a process designed under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, to provide reasonable assurance, not absolute assurance, regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States of America.

There are inherent limitations to the effectiveness of any system of internal control over financial reporting. These limitations include the possibility of human error, the circumvention or overriding of the system and reasonable resource constraints. Because of its inherent limitations, our internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate. Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework *Internal Control - Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

As of September 30, 2020, our management assessed the effectiveness of our internal control over financial reporting using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in *Internal Control-Integrated Framework (2013 Framework)*. Based on this assessment, our management concluded that, as of September 30, 2020, our internal control over financial reporting was effective based on those criteria.

Changes in Internal Control over Financial Reporting

Except as otherwise described above under Management's Report on Internal Control over Financial Reporting, there were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the fiscal 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

From time to time we may become involved in legal proceedings or be subject to claims arising in the ordinary course of our business. We are not presently a party to any legal proceedings that in the opinion of our management, if determined adversely to us, would have a material adverse effect on our business, financial condition, operating results or cash flows. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

ITEM 1A. RISK FACTORS

You should carefully consider the following risk factors, as well as the other information in this Quarterly Report on Form 10Q, and in our other public filings. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10Q and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risks Related to our Financial Condition, Integration and Capital Requirements

We have incurred losses since our inception, have a limited operating history on which to assess our business, and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a clinical development-stage biopharmaceutical company with a limited operating history. We have incurred net losses in each year since our inception. For the nine months ended September 30, 2020 and 2019, we reported a net loss of \$46.2 million and \$53.3 million, respectively. As of September 30, 2020, we had an accumulated deficit of \$287.7 million. Our prior losses, combined with expected future losses, have had and may continue to have an adverse effect on our stockholders' equity and working capital.

We believe that our currently available resources will be sufficient to fund our planned operations for at least the next 12 months following the issuance date of these unaudited condensed consolidated financial statements. We will continue to require substantial additional capital to continue our clinical development, manufacturing and regulatory approval efforts and potential commercialization activities. Accordingly, we will need to raise substantial additional capital to continue to fund our operations. The amounts and timing of our future funding requirements will depend on many factors, including our ability to achieve regulatory approval and the pace and results of our clinical development efforts. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates.

We have devoted substantially all of our financial resources to identify, acquire, and develop our product candidates, including manufacturing of clinical supplies, conducting clinical studies and providing general and administrative support for our operations. To date, we have financed our operations primarily through the sale of equity securities and debt facilities. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations, or grants. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We expect losses to increase as regulatory reviews of our NDA and MAA of lonafarnib in Progeria and Progeroid Laminopathies progress towards potential approvals and as we advance our clinical development programs in various clinical studies, particularly the D-LIVR pivotal study to support the submission of an NDA for lonafarnib for use in a hepatitis D virus indication. We may need significant additional resources in order to aggressively move lonafarnib forward successfully based on the discussions with the FDA. It may be several years, if ever, before we complete pivotal clinical studies and have a product candidate approved for commercialization. We expect to invest significant funds into our clinical candidates to advance these compounds to potential regulatory approval.

If we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates may receive approval, and our ability to achieve sufficient market acceptance, pricing, reimbursement from third-party payors, and adequate market share for our product candidates in those markets. Even if we obtain adequate market share for our product candidates, because the potential markets in which our product candidates may ultimately receive regulatory approval could be very small, we may never become profitable despite obtaining such market share and acceptance of our products. We have also agreed with The Progeria Research Foundation to make lonafarnib available to Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies patients under an Expanded Access Program that may not result in payment to us.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future and our expenses will increase substantially if and as we:

- continue the clinical development of our product candidates;
- in-license or acquire additional product candidates;
- undertake the manufacturing or have manufactured our product candidates;
- advance our programs into larger, more expensive clinical studies;
- initiate additional nonclinical, clinical, or other studies for our product candidates;
- identify and develop potential commercial opportunities, such as lonafarnib boosted with ritonavir, lonafarnib for the treatment of Progeria and Progeroid Laminopathies, lambda for HDV, and avexitide for PBH and CHI;
- seek regulatory and marketing approvals and reimbursement for our product candidates;
- establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval and market ourselves;
- seek to identify, assess, acquire, and/or develop other product candidates;
- make milestone, royalty or other payments under third-party license agreements;
- develop and educate HDV markets;
- seek to maintain, protect, and expand our intellectual property portfolio;
- seek to attract and retain skilled personnel;
- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with the development and potential for regulatory approval of our clinical candidates such as safety issues, clinical trial accrual delays, longer follow-up for planned studies, additional major studies, or supportive studies necessary to support marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a representative indication of our future performance.

We have never generated any revenue from product sales and may never be profitable.

We have no products approved for commercialization and have never generated any revenue. Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize, one or more of our product candidates. We do not anticipate generating significant revenue from product sales for the foreseeable future. Our ability to generate future revenue from product sales depends heavily on our success in many areas, including but not limited to:

- completing research and development of our product candidates;
- obtaining regulatory and marketing approvals for our product candidates;
- manufacturing product candidates and establishing and maintaining supply and manufacturing relationships with third parties that meet regulatory requirements and our supply needs in sufficient quantities to meet market demand for our product candidates, if approved;
- marketing, launching and commercializing product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;
- gaining market acceptance of our product candidates as treatment options;
- addressing any competing products;
- protecting and enforcing our intellectual property rights, including patents, trade secrets, and know-how;
- negotiating favorable terms in and maintaining any collaboration, licensing, or other arrangements into which we may enter;
- obtaining reimbursement or pricing for our product candidates that supports profitability; and
- attracting, hiring, and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our current pipeline of product candidates has been in-licensed from third parties and we will have to develop or acquire manufacturing capabilities in order to continue development and potential commercialization of our product candidates. Additionally, if we are not able to generate revenue from the sale of any approved products, we may never become profitable.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights.

To the extent that we raise additional capital through the sale of equity, debt or other securities convertible into equity, your ownership interest will be diluted, and the terms of these new securities may include liquidation or other preferences that adversely affect your rights as a common stockholder such as the Loan and Security Agreement we entered into with Oxford Finance LLC (Oxford Finance) in December 2016 (the Oxford Loan). The Oxford Loan was a \$25.0 million debt financing arrangement with Oxford Finance wherein we borrowed the first tranche of \$15.0 million upon closing of the debt financing in December 2016. In May 2018, we entered into an amendment to the Oxford Loan and borrowed \$5.0 million. In August 2018, we drew the final \$5.0 million upon achievement of certain clinical milestones. In March 2019, we entered into the third amendment to the Oxford Loan to refinance our outstanding principal balance of \$23.3 million. Upon refinancing, the Oxford Loan was increased to \$35.0 million in aggregate commitments, of which \$30.0 million in principal is outstanding. The Oxford Loan is secured by perfected first priority liens on our assets, excluding intellectual property but including a commitment by us to not allow any liens to be placed upon such intellectual property. The Oxford Loan includes customary events of default, including failure to pay amounts due, breaches of covenants and warranties, material adverse effect events, certain cross defaults and judgments, and insolvency.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates or future revenue streams or grant licenses on terms that are not favorable to us. Our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic. We cannot assure you that we will be able to obtain additional funding if and when necessary to fund our entire portfolio of product candidates to meet our projected plans. If we are unable to obtain funding on a timely basis, we may be required to delay or discontinue one or more of our development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on potential business opportunities, which could materially affect our business, financial condition, and results of operations.

Covenants in the Oxford Loan restrict our business and operations in many ways and if we do not effectively comply with our covenants, our financial conditions and results of operations could be adversely affected.

The Oxford Loan provides for up to \$35.0 million in term loans due on March 1, 2024, of which \$30.0 million in principal is outstanding. All of our current and future assets, except for intellectual property, secure our borrowings under the Oxford Loan. The Oxford Loan requires that we comply with certain covenants applicable to us, including among other things, covenants restricting dispositions, changes in business, management, ownership or business locations, mergers or acquisitions, indebtedness, encumbrances, distributions, investments, transactions with affiliates and subordinated debt, any of which could restrict our business and operations, particularly our ability to respond to changes in our business or to take specified actions to take advantage of certain business opportunities that may be presented to us. Our failure to comply with any of the covenants could result in a default under the Oxford Loan, which could permit the lenders to declare all or part of any outstanding borrowings to be immediately due and payable. If we are unable to repay those amounts, the lenders under the Oxford Loan could proceed against the collateral granted to them to secure that debt, and our inability to use or dispose of those assets would seriously harm our business. In addition, should we be unable to comply with these covenants or if we default on any portion of our outstanding borrowings, the lenders can also impose a 5.0% penalty, restrict access to additional borrowings under the loan and security agreement, and accelerate the maturity of the debt. Any default under the Oxford Loan would materially affect our liquidity and ability to fund our operations and complete our planned clinical trials and regulatory filings would be substantially impaired.

If the London Inter-Bank Offered Rate, or LIBOR, is discontinued, interest payments under the Oxford Loan may be calculated using another reference rate.

In July 2017, the Chief Executive of the United Kingdom Financial Conduct Authority (FCA), which regulates LIBOR, announced that the FCA intends to phase out the use of LIBOR by the end of 2021. In addition, the U.S. Federal Reserve, in conjunction with the Alternative Reference Rates Committee, a steering committee comprised of large U.S. financial institutions, is considering replacing U.S. dollar LIBOR with the Secured Overnight Financing Rate, or SOFR, a new index calculated by short-term repurchase agreements, backed by Treasury securities. Although there have been certain issuances utilizing SOFR, it is unknown whether this or any other alternative reference rate will attain market acceptance as a replacement for LIBOR. LIBOR is used as a benchmark rate throughout the Oxford Loan, and our credit agreement does not provide fallback language for all circumstances in which LIBOR ceases to be published. There remains uncertainty regarding the future utilization of LIBOR and the nature of any replacement rate, and any potential effects of the transition away from LIBOR on us are not known. The transition process may involve, among other things, increased volatility and illiquidity in markets for instruments that currently rely on LIBOR and may result in increased borrowing costs, the effectiveness of related transactions such as hedges, uncertainty under applicable documentation, including the Oxford Loan, or difficult and costly processes to amend such documentation. As a result, our ability to refinance our credit agreement or other indebtedness or to hedge our exposure to floating rate instruments may be impaired, which would adversely affect the operations of our business.

Risks Related to the Development of our Product Candidates

We are dependent on the success of our product candidates, which are in various stages of clinical development. Certain of our product candidates have produced results in academic settings to date or for other indications than those that we contemplate, and we cannot give any assurance that we will generate data for any of our product candidates sufficient to receive regulatory approval in our planned indications, which will be required before they can be commercialized.

To date, we have invested substantially all of our efforts and financial resources to identify, acquire, and develop our portfolio of product candidates. Our future success is dependent on our ability to successfully further develop, obtain regulatory approval for, and commercialize one or more of these product candidates. In May 2020, the FDA accepted our NDA for lonafarnib for treatment of Progeria and Progeroid Laminopathies with Priority Review and a PDUFA target action date of November 20, 2020. Our MAA is currently under standard review with the EMA as well. We currently generate no revenue from sales of any drugs, and we may never be able to develop or commercialize a product candidate. In addition, to the extent that we receive regulatory approval for lonafarnib in Progeria and Progeroid Laminopathies, we expect our commitment to provide access for patients with Progeria and Progeroid Laminopathies for no or minimal cost to those patients to result in a loss to us.

With respect to potential commercial products, we currently have one product candidate that is in Phase 3 clinical development and two development programs focused on two separate indications that we believe have completed Phase 2 and are advancing towards Phase 3. It may be years before our studies are initiated and completed, if at all.

We provide our geographically diverse clinical sites with good clinical practice protocols. We review and monitor the execution of our protocols at our various sites in an effort to understand those protocols are being followed. There can be no assurance that the data we develop for our product candidates in our planned indications will be sufficient or complete enough to obtain regulatory approval.

We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. We cannot be certain that any of our product candidates will be successful in clinical studies or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical studies. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

We may not be able to obtain FDA approval of our NDA for lonafarnib or any future NDA for our product candidates.

The clinical development, manufacturing, labeling, packaging, storage, recordkeeping, advertising, promotion, export, import, marketing and distribution and other possible activities relating to lonafarnib, lambda, avexitide and any other product candidate that we may develop in the future are subject to extensive regulation in the United States. Prior to the recent filing of our NDA for lonafarnib for the treatment of Progeria and Progeroid Laminopathies, we had not submitted an application for approval or obtained FDA approval for any product. This lack of experience may impede our ability to obtain FDA approval in a timely manner. We may receive requests for additional information during FDA's review of the NDA. The FDA may extend or be unable to meet its PDUFA goal date for the NDA, and may issue a complete response letter, rather than an approval.

Approval of an NDA is not guaranteed, and the approval process is an expensive and uncertain process that may take several years. The FDA and foreign regulatory entities also have substantial discretion in the approval process. The number and types of preclinical studies and clinical trials that will be required for approval varies depending on the product candidate, the disease or the condition that the product candidate is designed to target and the regulations applicable to any particular product candidate. Data are subject to varying interpretation and the FDA may not agree that our clinical data support that any of our product candidates are safe and effective for the proposed therapeutic use. Despite the time and expense associated with preclinical studies and clinical trials, failure can occur at any stage, and we could encounter problems that require us to repeat or perform additional preclinical studies or clinical trials or generate additional chemistry, manufacturing and controls data, including drug product stability data. HGPS and PL patients have accelerated or extensive cardiovascular disease. In previous studies, ECG abnormalities were reported, and were also observed in our Ionafarnib program. We do not believe this will impact the approval, but we expect it to be reflected in the product labeling for HGPS and PL. We do not expect that this will impact the conduct of the D-LIVR HDV study. The FDA and similar foreign authorities could delay, limit or deny approval of a product candidate, and may ultimately approve the product for narrower indications or with unfavorable labeling that would impede our commercialization of the drug.

Approval procedures vary among countries and can involve additional product testing and additional administrative review periods, including obtaining reimbursement and pricing approval in select markets. The time required to obtain approval in other countries might differ from that required to obtain FDA approval. The regulatory approval process in other countries may include all of the risks associated with FDA approval as well as additional, presently unanticipated, risks. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others, including the risk that our product candidates may not be approved for all indications requested and that such approval may be subject to limitations on the indicated uses for which the product may be marketed.

Our business strategy is based upon obtaining and maintaining Orphan Drug designation for our product candidates, which is an uncertain process. The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, and inherently unpredictable. If we are unable to obtain Orphan Drug designation or regulatory approval for our product candidates, our business would be substantially harmed.

Our approach to identifying and developing product candidates depends, in large part, on our ability to obtain and maintain Orphan Drug designation from regulatory authorities in major markets. Without the potential protection of this regulatory exclusivity upon approval, many of our product candidates would otherwise not justify investment. While we assess the potential for obtaining Orphan Drug designation at the time that we contemplate the acquisition of product candidates and we intend to timely file for such designation, there can be no assurance that we will obtain Orphan Drug designation or be able to successfully meet the regulatory requirements to maintain that designation with the planned clinical trials for our product candidates. Failure to obtain and maintain Orphan Drug designation would make our product candidates significantly less competitive and potentially not viable investments for further development. Although we currently have Orphan Drug designation for some of our product candidates in multiple targeted indications, failure to demonstrate significant benefit over existing approved drugs in pivotal clinical trials may lead to marketing approval but without qualifying for Orphan Drug protection in some regions, such as in Europe.

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable, typically takes many years following the commencement of clinical studies and depends upon numerous factors. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. We have not obtained regulatory approval for any product candidate, and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Applications for our product candidates could fail to receive regulatory approval for many reasons, including but not limited to the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design, size or implementation of our clinical studies;
- the population studied in the clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from our development efforts;
- the data collected from clinical studies of our product candidates may not be sufficient or complete or meet the regulatory requirements to support the submission of a new drug application (NDA) or other submission or to obtain regulatory approval in the United States or foreign jurisdictions;

- the FDA or comparable foreign regulatory authorities may find failures in our manufacturing processes, validation procedures and specifications, or facilities of our third-party manufacturers with which we contract for clinical and commercial supplies that may delay or limit our ability to obtain regulatory approval for our product candidates; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our NDA or other submission insufficient for approval.

The lengthy and uncertain regulatory approval process, as well as the unpredictability of the results of clinical studies, may result in our failing to obtain regulatory approval to market any of our product candidates or to be significantly delayed from our expectations for potential approval, which would significantly harm our business, results of operations, and prospects. In addition, although we have obtained Orphan Drug designation for five of our development programs to date, there can be no assurance that the FDA will grant our similar status for our other proposed development indications or other product candidates in the future.

Although the FDA has granted Rare Pediatric Disease designation to lonafarnib for the treatment of Progeria and Progeroid Laminopathies and to avexitide for the treatment of congenital hyperinsulinism, NDA approvals for these programs may not meet the eligibility criteria for a priority review voucher.

Our lonafarnib and avexitide compounds have received Rare Pediatric Disease (RPD) designation from the FDA for the treatment of Progeria and Progeroid Laminopathies and Congenital Hyperinsulinism (CHI), respectively. The FDA defines a “rare pediatric disease” as a disease that affects fewer than 200,000 individuals in the United States primarily under the age of 18 years. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a “rare pediatric disease” may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval. In addition, the priority review voucher is only awarded to an NCE, thus if a compound is approved first for an indication that is not a rare pediatric disease the compound may not be eligible to receive the voucher.

Congress has only authorized the Rare Pediatric Disease Priority Review Voucher program until September 30, 2020. However, if a drug candidate receives Rare Pediatric Disease designation before October 1, 2020, it is eligible to receive a voucher if it is approved before October 1, 2022. Lonafarnib or avexitide may not be approved by that date, or at all, and, therefore, we may not be in a position to obtain a priority review voucher prior to expiration of the program, unless Congress further reauthorizes the program. In addition, we have agreed to equally share with the Progeria Research Foundation any proceeds from the sale of a Priority Review Voucher that we may receive as the sponsor of a rare pediatric disease product application for lonafarnib for the treatment of progeria.

There is no assurance we will receive a Rare Pediatric Disease Priority Review Voucher. Also, although Priority Review Vouchers may be sold or transferred to third parties, there is no guarantee that we will be able to realize any value if we were to sell a Priority Review Voucher.

Although we have received Breakthrough Therapy designation for four of our development programs, this may not lead to a faster development, regulatory review or approval process, and it does not increase the likelihood that any of our product candidates will receive marketing approval in the United States.

We have received Breakthrough Therapy designation for lonafarnib and lambda for the treatment of HDV, lonafarnib for the treatment of Progeria and Progeroid Laminopathies and for avexitide for the treatment of post-bariatric hypoglycemia. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval. The Breakthrough Therapy designations we have obtained may not result in faster development processes, reviews or approvals compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, the FDA may later decide that any of our development programs no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies may not be predictive of future study results.

Clinical testing is expensive and generally takes many years to complete, and the outcome is inherently uncertain. Failure can occur at any time during the clinical study process. The results of preclinical studies and early clinical studies of our product candidates may not be predictive of the results of larger, later-stage controlled clinical studies. Product candidates that have shown promising results in early-stage clinical studies may still suffer significant setbacks in subsequent clinical studies. Our clinical studies to date have been conducted on a small number of patients in limited numbers of clinical sites and in academic settings or for other indications. We will have to conduct larger, well-controlled studies in our proposed indications to verify the results obtained to date and to support any regulatory submissions for further clinical development. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical studies due to lack of efficacy or adverse safety profiles despite promising results in earlier, smaller clinical studies. Moreover, clinical data are often susceptible to varying interpretations and analyses. We do not know whether any Phase 2, Phase 3, or other clinical studies we have conducted or may conduct will demonstrate consistent or adequate efficacy and safety with respect to the proposed indication for use sufficient to obtain regulatory approval to receive regulatory approval or market our drug candidates. For example, in 2018 we announced negative results from two of our Phase 2 clinical trials of ubenimex in two different indications and as a result we have terminated further development of ubenimex.

We may find it difficult to enroll patients in our clinical studies given the limited number of patients who have the diseases for which our product candidates are being studied. Difficulty in enrolling patients could delay or prevent clinical studies of our product candidates.

Identifying and qualifying patients to participate in clinical studies of our product candidates is essential to our success. The timing of our clinical studies depends in part on the rate at which we can recruit patients to participate in clinical trials of our product candidates, and we may experience delays in our clinical studies if we encounter difficulties in enrollment.

The eligibility criteria of our planned clinical studies may further limit the available eligible study participants as we expect to require that patients have specific characteristics that we can measure or meet the criteria to assure their conditions are appropriate for inclusion in our clinical studies. We may not be able to identify, recruit, and enroll a sufficient number of patients to complete our clinical studies in a timely manner because of the perceived risks and benefits of the product candidate under study, the availability and efficacy of competing therapies and clinical studies, and the willingness of physicians to participate in our planned clinical studies. Additionally, we may experience delays in patient enrollment for our clinical trials as a result of the evolving COVID-19 global pandemic and competition for patients at our European clinical trial sites due to the recent conditional approval of Hepcludex in Europe. For example, certain clinical study sites that were scheduled to open have been delayed in activating and other sites have suspended randomization of subjects and if this continues longer than anticipated, the D-LIVR trial may be delayed further than anticipated. If patients are unwilling to participate in our clinical studies for any reason, the timeline for conducting studies and obtaining regulatory approval of our product candidates may be delayed.

If we experience delays in the completion of, or termination of, any clinical study of our product candidates, the commercial prospects of our product candidates could be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. In addition, any delays in completing our clinical studies would likely increase our overall costs, impair product candidate development and jeopardize our ability to obtain regulatory approval relative to our current plans. Any of these occurrences may harm our business, financial condition, and prospects significantly.

Clinical studies are costly, time consuming and inherently risky, and we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Clinical development is expensive, time consuming and involves significant risk. We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical studies can occur at any stage of development. Events that may prevent successful or timely completion of clinical development include but are not limited to:

- inability to generate satisfactory preclinical, toxicology, or other in vivo or in vitro data or diagnostics to support the initiation or continuation of clinical studies necessary for product approval;
- delays in reaching agreement on acceptable terms with contract research organizations (CROs) and clinical study sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical study sites;
- delays in obtaining required Institutional Review Board (IRB) approval at each clinical study site;
- failure to permit the conduct of a study by regulatory authorities, after review of an investigational new drug (IND) or equivalent foreign application or amendment;

- delays in recruiting qualified patients, or patients dropping out of, in our clinical studies, including as a result of the evolving COVID-19 global pandemic;
- failure by clinical sites or our CROs or other third parties to adhere to clinical study requirements or report complete findings;
- failure to perform the clinical studies in accordance with the FDA's GCP requirements, or applicable foreign regulatory guidelines;
- occurrence of adverse events associated with our product candidates;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- the cost of clinical studies of our product candidates;
- negative or inconclusive results from our clinical trials which may result in our deciding, or regulators requiring us, to conduct additional clinical studies or abandon development programs in other ongoing or planned indications for a product candidate; and
- delays in reaching agreement on acceptable terms with third-party manufacturers and the time for manufacture of sufficient quantities of our product candidates for use in clinical studies.

Any inability to successfully complete clinical development and obtain regulatory approval could result in additional costs to us or impair our ability to generate revenue. Clinical study delays could also shorten any periods during which our products have patent protection and may allow competitors to develop and bring products to market before we do, which could impair our ability to obtain Orphan Drug designation exclusivity and to successfully commercialize our product candidates and may harm our business and results of operations.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Our lonafarnib product candidate has been studied in thousands of oncology patients and the most common non-hematologic adverse events of any grade were gastrointestinal system disorders (nausea, anorexia, diarrhea and vomiting), weight loss, fatigue and rash. Our lonafarnib product candidate for HGPS and PL has been reported to cause ECG abnormalities, but these ECG abnormalities have not resulted in a risk of mortality for these patients. There is no guarantee that additional or more severe side effects or other properties will not be identified through ongoing clinical studies by other uses of lonafarnib for other indications or our own clinical trials. Our lambda product candidate is well-characterized and has been studied in thousands of HBV and HCV patients and the most common adverse events seen are moderate headache, pyrexia, fatigue, and myalgia. ALT flares that were seen result from vigorous antiviral immunological response to treatment, not due to direct hepatotoxicity. There is no guarantee that additional or more severe side effects will not be identified through ongoing clinical studies for other uses of lambda. Undesirable side effects, other properties, and negative results for other indications may negatively impact the development and potential for approval of our product candidates for our proposed indications. For example, the ECG abnormalities seen with lonafarnib in HGPS and PL patients has the potential to impact the labeling for lonafarnib boosted with ritonavir for HDV. Our avexitide product candidate has been studied in 54 PBH patients and 39 CHI patients and the most common adverse events are injection site bruising, nausea, and headache. There is no guarantee that additional or more severe side effects will not be identified through ongoing clinical studies for other uses of avexitide in clinical trials.

Additionally, even if one or more of our product candidates receives marketing approval, and we or others later may identify undesirable side effects caused by such products, potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may withdraw approvals of such products;
- regulatory authorities may require additional warnings on the label;
- we may be required to create a Risk Evaluation and Mitigation Strategy (REMS) plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of a product candidate, even if approved, and could significantly harm our business, results of operations, and prospects.

Even if we obtain regulatory approval for a product candidate, we will remain subject to ongoing regulatory requirements.

If our product candidates are approved, they will be subject to ongoing regulatory requirements with respect to manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy and other post-approval information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to continuously comply with FDA and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practices (cGMP) regulations and corresponding foreign regulatory manufacturing requirements. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA or MAA.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product candidate may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. We will be required to report certain adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities.

Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance. If our original marketing approval for a product candidate was obtained through an accelerated approval pathway, we could be required to conduct a successful post-marketing clinical study to confirm the clinical benefit for our products. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing, or labeling of a product, the regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any of our ongoing clinical studies;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities; or
- require a product recall.

In addition, prescription drugs may be promoted only for the approved indications in accordance with the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. However, physicians may, in their independent medical judgment, prescribe legally available products for off-label uses. The FDA does not regulate the behavior of physicians in their choice of treatments, but the FDA does restrict manufacturer's communications on the subject of off-label use of their products.

Any government investigation of alleged violations of law would be expected to require us to expend significant time and resources in response and could generate adverse publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to develop and commercialize our products and the value of us and our operating results would be adversely affected.

We rely on third parties to conduct our clinical studies, manufacture our product candidates and perform other services. If these third parties do not successfully perform and comply with regulatory requirements, we may not be able to successfully complete clinical development, obtain regulatory approval or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon investigators and third-party CROs to conduct, monitor and manage our ongoing clinical programs. We rely on these parties for execution of clinical studies and manage and control only certain aspects of their activities. We remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We, our investigators, and our CROs and other vendors are required to comply all applicable laws, regulations and guidelines, including those required by the FDA and comparable foreign regulatory authorities for all of our product candidates in clinical development. If we or any of our investigators, CROs or vendors fail to comply with applicable laws, regulations and guidelines, the results generated in our clinical studies may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional studies before approving our marketing applications. We cannot assure you that our CROs and other vendors will meet these requirements, or that upon inspection by any regulatory authority, such regulatory authority will determine that efforts, including any of our clinical studies, comply with applicable requirements. Our failure to comply with these laws, regulations and guidelines may require us to repeat clinical studies or conduct larger additional studies, which would be costly and delay the regulatory approval process.

If any of our relationships with investigators or third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs in a timely manner or do so on commercially reasonable terms. In addition, our CROs may not prioritize our clinical studies relative to those of other customers and any turnover in personnel or delays in the allocation of CRO employees by the CRO may negatively affect our clinical studies. If investigators or CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, our clinical studies may be delayed or terminated, and we may not be able to meet our current plans with respect to our product candidates. CROs may also involve higher costs than anticipated, which could negatively affect our financial condition and operations.

In addition, we do not currently have, nor do we plan to establish, the capability to manufacture product candidates for use in the conduct of our clinical studies or in support of our commercialization of potential products, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale without the use of third-party manufacturers. We plan to rely on third-party manufacturers and their responsibilities will include purchasing from third-party suppliers the materials necessary to produce our product candidates for our clinical studies and regulatory approval. There are expected to be a limited number of suppliers for the active ingredients and other materials that we expect to use to manufacture our product candidates, and we may not be able to identify alternative suppliers to prevent a possible disruption of the manufacture of our product candidates for our clinical studies, and, if approved, ultimately for commercial sale. Although we generally do not expect to begin a clinical study unless we believe we have a sufficient supply of a product candidate to complete the study, any significant delay or discontinuity in the supply of a product candidate, or the active ingredient or other material components in the manufacture of the product candidate, could delay completion of our clinical studies and potential timing for regulatory approval of our product candidates, which would harm our business and results of operations.

With respect to our lonafarnib program, we procured an inventory of product from Merck to supply our initial clinical study needs. In 2016, we transferred the manufacturing of drug substance and drug product to our third-party contractors. The material used for lonafarnib HDV pivotal trials and Progeria clinical studies are sourced from Eiger-controlled CMOs. These same vendors are currently under development for commercial qualification. Materials used for our avexitide clinical trials are also sourced from CMOs. Our vendors have successfully made GMP batches for our clinical studies. If these CMOs are not able to provide us with sufficient quantities of drug substance and drug product for our clinical trials or in support of our commercialization of potential products on a timely basis, or at all, whether due to production shortages or other supply interruptions resulting from the ongoing COVID-19 pandemic or otherwise, our clinical trials or regulatory approval may be delayed, or could impair our ability to generate revenue from the sale of such product candidate.

We rely and expect to continue to rely on third parties to manufacture our clinical product supplies, and if those third parties fail to obtain approval of government regulators, fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices our product candidates could be stopped, delayed, or made less profitable.

We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture our clinical supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on outside vendors to source raw materials and manufacture our clinical supplies of our product candidates and plan to continue relying on third parties to manufacture our product candidates on a commercial scale, if approved.

The facilities used by our contract manufacturers to manufacture our product candidates will be subject to pre-approval inspection by the FDA that will be conducted after we submit our marketing applications to the FDA or comparable foreign regulatory authorities. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements, known as cGMPs, for manufacture of our product candidates. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, our NDA and MAA for lonafarnib and future applications may not be approved by regulatory authorities, which would significantly delay our commercialization plans and increase our costs. We have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

We do not yet have sufficient information to reliably estimate the cost of the commercial manufacturing of our product candidates, and the actual cost to manufacture our product candidates could materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product.

In addition, our reliance on third-party manufacturers exposes us to the following additional risks:

- We may be unable to identify manufacturers on acceptable terms or at all;
- Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any;
- Contract manufacturers may not be able to execute our manufacturing procedures appropriately;
- Our future contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store and distribute our products;
- Manufacturers are subject to ongoing periodic unannounced inspection by the FDA and corresponding state agencies to ensure strict compliance with cGMP and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards;
- We may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our product candidates; and
- Our third-party manufacturers could breach or terminate their agreement with us.

Each of these risks could delay our clinical trials, the approval of any of our product candidates by the FDA or comparable foreign regulatory authorities or the commercialization of our product candidates or result in higher costs or deprive us of potential product revenue. In addition, we rely on third parties to perform release testing on our product candidates prior to delivery to patients. If these tests are not conducted appropriately and test data is not reliable, patients could be put at risk of serious harm and could result in product liability suits.

The manufacturing of medical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of biologic products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if contaminants are discovered in our supply of our product candidates or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot assure you that any stability or other issues relating to the manufacture of our product candidates will not occur in the future. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidates to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

If the market opportunities for our product candidates are smaller than we believe they are, we may not meet our revenue expectations and, even assuming approval of a product candidate, our business may suffer. Because the patient populations in the market for our product candidates may be small, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth.

We focus our product development principally on treatments for rare and ultra-rare diseases. Given the small number of patients who have the diseases that we are targeting, our eligible patient population and pricing estimates may differ significantly from the actual market addressable by our product candidate. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. For example, for lonafarnib and lambda, HDV is associated with hepatitis B virus infection, which is a pre-requisite for the replication of HDV. Although we believe that the data are supportive of antiviral activity against HDV, there can be no assurance that our clinical trials will successfully address this condition. Likewise, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Moreover, if lonafarnib receives regulatory approval for use in Progeria and Progeroid Laminopathies, we expect that the sales of lonafarnib to patients with Progeria and Progeroid Laminopathies will have limited profits.

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are similar, more advanced, or more effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. We are currently aware of various existing therapies that may compete with our product candidates. For example, we have competitors both in the United States and internationally, including multinational pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies. Some of the pharmaceutical and biotechnology companies we expect to compete with include Gilead Sciences, Merck, Roche, Holding AG, Actelion Pharmaceuticals US, Inc., Johnson & Johnson, Replicor, Inc., Myr, Arrowhead Pharmaceuticals, Novartis International AG, Zealand Pharmaceuticals, Xeris Pharmaceuticals, and Rezolute, Inc. as well as other smaller companies or biotechnology startups and large multinational pharmaceutical companies. Many of our competitors have substantially greater financial, technical, and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. As a result, these companies may obtain regulatory approval more rapidly than we are able to and may be more effective in selling and marketing their products as well. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring, or licensing on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop, or achieve earlier patent protection, regulatory approval, product commercialization, and market penetration than we do. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

We currently have limited marketing and sales experience. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

Although certain of our employees may have marketed, launched and sold other pharmaceutical products in the past while employed at other companies, we have no recent experience selling and marketing our product candidates and we currently have no marketing or sales organization. To successfully commercialize any products that may result from our development programs, we will need to invest in and develop these capabilities, either on our own or with others, which would be expensive, difficult and time consuming. Any failure or delay in the timely development of our internal commercialization capabilities could adversely impact the potential for success of our products.

Further, given our lack of prior experience in marketing and selling biopharmaceutical products, we may rely on future collaborators to commercialize our products. If collaborators do not commit sufficient resources to commercialize our future products and we are unable to develop the necessary marketing and sales capabilities on our own, we will be unable to generate sufficient product revenue to sustain or grow our business. We may be competing with companies that currently have extensive and well-funded marketing and sales operations, in particular in the markets our product candidates are intended to address. Without appropriate capabilities, whether directly or through third-party collaborators, we may be unable to compete successfully against these more established companies. In addition, we have established an expanded access program in order to make lonafarnib available for patients with Progeria and Progeroid Laminopathies, which requires additional resources and costs to support.

The commercial success of any of our current or future product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

Even with the approvals from the FDA and comparable foreign regulatory authorities, the commercial success of our products will depend in part on the health care providers, patients, and third-party payors accepting our product candidates as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors and other health care providers. The degree of market acceptance of any of our products will depend on a number of factors, including without limitation:

- the efficacy of the product as demonstrated in clinical studies and potential advantages over competing treatments;
- the prevalence and severity of the disease and any side effects;
- the clinical indications for which approval is granted, including any limitations or warnings contained in a product's approved labeling;
- the convenience and ease of administration;
- the cost of treatment;
- the willingness of the patients and physicians to accept these therapies;
- the marketing, sales and distribution support for the product;
- the publicity concerning our products or competing products and treatments; and
- the pricing and availability of third-party insurance coverage and reimbursement.

Even if a product displays a favorable efficacy and safety profile upon approval, market acceptance of the product remains uncertain. Efforts to educate the medical community and third-party payors on the benefits of the products may require significant investment and resources and may never be successful. If our products fail to achieve an adequate level of acceptance by physicians, patients, third-party payors, and other health care providers, we will not be able to generate sufficient revenue to become or remain profitable.

Failure to obtain or maintain adequate reimbursement or insurance coverage for new or current products could limit our ability to market those products and decrease our ability to generate revenue.

The pricing, coverage and reimbursement of our products must be sufficient to support our commercial efforts and other development programs and the availability and adequacy of coverage and reimbursement by governmental and private payors are essential for most patients to be able to afford expensive treatments, particularly in Orphan Drug designated indications where the eligible patient population is small. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid for or reimbursed by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or government authorities, private health insurers, and other third-party payors. If coverage and reimbursement are not available, or are available only in limited amounts, we may have to subsidize or provide products for free or we may not be able to successfully commercialize our products. For example, lonafarnib for patients with Progeria and Progeroid Laminopathies provided under an Expanded Access Program may not result in reimbursement.

In addition, there is significant uncertainty related to the insurance coverage and reimbursement for newly approved products. In the United States, the principal decisions about coverage and reimbursement for new drugs are typically made by the Centers for Medicare & Medicaid Services (CMS), an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new drug will be covered and reimbursed under Medicare. Private payors tend to follow the coverage reimbursement policies established by CMS to a substantial degree. It is difficult to predict what CMS will decide with respect to reimbursement for products such as ours and what reimbursement codes our products may receive.

Outside the United States, international operations are generally subject to extensive governmental price controls and other price-restrictive regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of products. In many countries, the prices of products are subject to varying price control mechanisms as part of national health systems. Price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our products. Accordingly, in markets outside the United States, the potential revenue may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to limit or reduce healthcare costs may result in restrictions on coverage and the level of reimbursement for new products and, as a result, they may not cover or provide adequate payment for our products. We expect to experience pricing pressures in connection with products due to the increasing trend toward managed healthcare, including the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs has and is expected to continue to increase in the future. As a result, profitability of our products may be more difficult to achieve even if they receive regulatory approval.

We intend to rely on a combination of exclusivity from Orphan Drug designation as well as patent rights for our product candidates and any future product candidates. If we are unable to obtain or maintain exclusivity from the combination of these approaches, we may not be able to compete effectively in our markets.

Our business strategy is to focus on product candidates for which Orphan Drug designation may be obtained in the major markets of the world. In addition, we rely or will rely upon a combination of patents, trade secret protection, and confidentiality agreements to protect the intellectual property related to our technologies and product candidates. For example, the portfolio of patents licensed from Merck expires before the anticipated launch date of lonafarnib. Our success depends in large part on our and our licensors' ability to obtain regulatory exclusivity and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and products.

Under the Orphan Drug Act, the FDA may designate a product as an Orphan Drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union (the EU), the EMA's Committee for Orphan Medicinal Products (COMP) grants Orphan Drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the EU. Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention, or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the United States, Orphan Drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. In addition, if a product receives the first FDA approval for the indication for which it has Orphan Drug designation, the product is entitled to Orphan Drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity. In the EU, Orphan Drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the Orphan Drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Because the extent and scope of patent protection for our products may in some cases be limited, Orphan Drug designation is especially important for our products for which Orphan Drug designation may be available. For eligible drugs, we plan to rely on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain Orphan Drug exclusivity for our drug products and biologic products that do not have broad patent protection, our competitors may then sell the same drug to treat the same condition sooner than if we had obtained Orphan Drug exclusivity and our revenue will be reduced.

Even though we have Orphan Drug designations for each of our development programs in the United States and Europe, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain Orphan Drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition. Even after an Orphan Drug is approved, the FDA or EMA can subsequently approve the same drug with the same active moiety for the same condition if the FDA or EMA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. Orphan Drug designation neither shortens the development time or regulatory review time of a product candidate nor gives the product candidate any advantage in the regulatory review or approval process.

We have sought to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates that are important to our business. This process is expensive and time consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unsolved. The patent applications that we own or in-licensed may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue, and even if such patents cover our product candidates, third parties may challenge their validity, enforceability, or scope, which may result in such patents being narrowed, found unenforceable or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates, or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

We, independently or together with our licensors, have filed several patent applications covering various aspects of our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

Although we have licensed a number of patents covering methods of use and certain compositions of matter, we do not have complete patent protection for our product candidates. For example, the patent coverage for the lonafarnib composition of matter expires before the anticipated launch date. Likewise, most of the patents or applications covering products that we have licensed in from Stanford have limited protection outside of the United States. Therefore, a competitor could develop the same or similar product that may compete with our product candidate.

Certain of our product licenses are limited to specified indications or therapeutic areas which may result in the same compound being developed and commercialized by a third party whom we have no control over or rights against. This may result in safety data, pricing or off label uses from that third party's product that may negatively affect the development and commercialization of our product candidates. If we cannot obtain and maintain effective protection of exclusivity from our regulatory efforts and intellectual property rights, including patent protection, for our product candidates, we may not be able to compete effectively, and our business and results of operations would be harmed.

We may not have sufficient patent term protections for our products to effectively protect our business.

Patents have a limited term. In the United States, the statutory expiration of a patent is generally 20 years after it is filed. Although various extensions may be available, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications. In addition, upon issuance in the United States any patent term can be adjusted based on certain delays caused by the applicant(s) or the United States Patent and Trademark Office (USPTO). For example, a patent term can be reduced based on certain delays caused by the patent applicant during patent prosecution.

Patent term extensions under the Hatch-Waxman Act in the United States and under supplementary protection certificates in Europe may be available to extend the patent or data exclusivity terms of products. With respect to lonafarnib, lambda and avexitide, a substantial portion of the potential commercial opportunity will likely rely on patent term extensions, and we cannot provide any assurances that any such patent term extensions will be obtained and, if so, for how long. As a result, we may not be able to maintain exclusivity for our products for an extended period after regulatory approval, which would negatively impact our business and results of operations. If we do not have sufficient patent terms or regulatory exclusivity to protect our products, our business and results of operations will be adversely affected.

Patent laws and rule changes could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. We therefore cannot be certain that it or our licensors were the first to make the invention claimed in our owned and licensed patents or pending applications, or that we or our licensor were the first to file for patent protection of such inventions. Assuming the other requirements for patentability are met, in the United States prior to March 15, 2013, the first to make the claimed invention is entitled to the patent, while outside the United States, the first to file a patent application is entitled to the patent. After March 15, 2013, under the Leahy-Smith America Invents Act (the Leahy-Smith Act) enacted on September 16, 2011, the United States has moved to a first to file system. The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and may also affect patent litigation. The effects of these changes are currently unclear as the USPTO must still implement various regulations, the courts have yet to address any of these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. In general, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

If we are unable to maintain effective proprietary rights for our product candidates or any future product candidates, we may not be able to compete effectively in our markets.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors, and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There have been many lawsuits and other proceedings involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, and reexamination proceedings before the USPTO and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are using or exploiting their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture, or methods for treatment related to the use or manufacture of our product candidates. Even if we conduct freedom to operate analyses, we would expect to do so only with respect to certain of our product candidates as they move through development. Accordingly, there may be third-party patents that would impair our ability to commercialize product candidates and we cannot assure you that we could obtain a license, or even if available, whether such license might be obtained on commercially reasonable terms. Even in those situations where we conduct a freedom to operate analysis, there can be no assurance that we would identify all relevant or necessary patents and patent applications that may apply to the manufacture and commercialization of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe, and if patents issue with respect to any such application and we become aware of such issuance, we would have to determine its impact on our efforts to develop and commercialize our product candidates and the strategy for obtaining a license or contesting any such issued patent.

If any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, the manufacturing process of any of our product candidates, methods of use, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire or are finally determined to be invalid or unenforceable. Such a license may not be available on commercially reasonable terms, or at all.

If we fail to obtain a license, then parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

We may not be successful in meeting our diligence obligations under our existing license agreements necessary to maintain our product candidate licenses in effect. In addition, if required in order to commercialize our product candidates, we may be unsuccessful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to the intellectual property, through licenses from third parties and under patents that we do not own, to develop and commercialize our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to maintain in effect these proprietary rights. For example, we have certain specified diligence obligations under our Stanford license agreement for lonafarnib. We may not be able to achieve the required diligence milestones in a timely manner, which may result in Stanford's right to terminate the license agreement, and we may be unable to successfully negotiate an extension or waiver of those termination rights. Any termination of license agreements with third parties with respect to our product candidates would be expected to negatively impact our business prospects.

We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. Even if we are able to license or acquire third-party intellectual property rights that are necessary for our product candidates, there can be no assurance that they will be available on favorable terms.

We collaborate with U.S. and foreign academic institutions to identify product candidates, accelerate our research and conduct development. Typically, these institutions have provided us with an option to negotiate an exclusive license to any of the institution's rights in the patents or other intellectual property resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue a program of interest to us.

If we are unable to successfully obtain and maintain rights to required third-party intellectual property, we may have to abandon development of that product candidate or pay additional amounts to the third party, and our business and financial condition could suffer.

Our product candidates may be subject to generic competition.

Under the Hatch-Waxman Act, a pharmaceutical manufacturer may file an abbreviated new drug application (ANDA) seeking approval of a generic copy of an approved innovator product. Under the Hatch-Waxman Act, a manufacturer may also submit an NDA under section 505(b)(2) that references the FDA's finding of safety and effectiveness of a previously approved drug. A 505(b)(2) NDA product may be for a new or improved version of the original innovator product. Innovative small molecule drugs may be eligible for certain periods of regulatory exclusivity (e.g., five years for new chemical entities, three years for changes to an approved drug requiring a new clinical study, seven years for Orphan Drugs), which preclude FDA approval (or in some circumstances, FDA filing and review of) an ANDA or 505(b)(2) NDA relying on the FDA's finding of safety and effectiveness for the innovative drug. In addition to the benefits of regulatory exclusivity, an innovator NDA holder may have patents claiming the active ingredient, product formulation or an approved use of the drug, which would be listed with the product in the FDA publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," known as the "Orange Book." If there are patents listed in the Orange Book, a generic applicant that seeks to market its product before expiration of the patents must include in the ANDA or 505(b)(2) what is known as a "Paragraph IV certification," challenging the validity or enforceability of, or claiming non-infringement of, the listed patent or patents. Notice of the certification must be given to the innovator, too, and if within 45 days of receiving notice the innovator sues to protect its patents, approval of the ANDA is stayed for 30 months, or as lengthened or shortened by the court.

If there are patents listed for our products in the Orange Book after approval by FDA, ANDAs and 505(b)(2) NDAs with respect to those products would be required to include a certification as to each listed patent indicating whether the ANDA applicant does or does not intend to challenge the patent. We cannot predict whether any patents issuing from our pending patent applications will be eligible for listing in the Orange Book, how any generic competitor would address such patents, whether we would sue on any such patents, or the outcome of any such suit.

We may not be successful in securing or maintaining proprietary patent protection in the United States and/or in other countries for products and technologies we develop or license. Moreover, if any patents that are granted and listed in the Orange Book are successfully challenged by way of a Paragraph IV certification and subsequent litigation, the affected product could more immediately face generic competition and its sales would likely decline materially. Should sales decline, we may have to write off a portion or all of the intangible assets associated with the affected product and our results of operations and cash flows could be materially and adversely affected.

The patent protection and patent prosecution for some of our product candidates is dependent on third parties.

While we normally seek and gain the right to fully prosecute the patents relating to our product candidates, there may be times when patents relating to our product candidates are controlled by our licensors. This is the case with our agreements with Stanford and Nippon Kayaku, each of whom is primarily responsible for the prosecution of patents and patent applications licensed to us under the applicable collaboration agreements. If they or any of our future licensors fail to appropriately and broadly prosecute and maintain patent protection for patents covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using, importing, and selling competing products. In addition, even where we now have the right to control patent prosecution of patents and patent applications, we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensors in effect from actions prior to us assuming control over patent prosecution.

If we fail to comply with obligations in the agreements under which we license intellectual property and other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license and supply agreements that are important to our business and expects to enter into additional license agreements in the future. Our existing agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, purchasing, supply and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, our agreements may be subject to termination by the licensor, in which event we would not be able to develop, manufacture or market products covered by the license or subject to supply commitments.

Although we are not currently involved in any intellectual property litigation, we may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. Although we are not currently involved in any intellectual property litigation, if we or one of our licensing partners were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable.

Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we have written agreements and make every effort to ensure that our employees, consultants and independent contractors do not use the proprietary information or intellectual property rights of others in their work forums, and we are not currently subject to any claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties, we may in the future be subject to such claims. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Risks Related to our Business Operations

Our future success depends in part on our ability to retain our President and Chief Executive Officer and to attract, retain, and motivate other qualified personnel.

We are highly dependent on David Cory, our President and Chief Executive Officer, the loss of whose services may adversely impact the achievement of our objectives. Mr. Cory could leave our employment at any time, as he is an “at will” employee. Recruiting and retaining other qualified employees, consultants, and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of highly qualified personnel in our industry, which is likely to continue. As a result, competition for personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in development and commercialization of our product candidates may make it more challenging to recruit and retain qualified personnel. The inability to recruit and retain qualified personnel, or the loss of the services of Mr. Cory, may impede the progress of our research, development, and commercialization objectives and would negatively impact our ability to succeed in our in-licensing strategy.

We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

As of September 30, 2020, we had 29 full-time employees. As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, manufacturing, sales, marketing, financial, legal, and other resources. Our management may need to divert a disproportionate amount of their attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Failure to comply with existing or future laws and regulations related to privacy or data security could lead to government enforcement actions (which could include civil or criminal fines or penalties), private litigation, other liabilities, and/or adverse publicity. Compliance or the failure to comply with such laws could increase the costs of our products and services, could limit their use or adoption, and could otherwise negatively affect our operating results and business.

Regulation of data processing is evolving, as federal, state, and foreign governments continue to adopt new, or modify existing, laws and regulations addressing data privacy and security, and the collection, processing, storage, transfer, and use of data. We and our partners may be subject to current, new, or modified federal, state, and foreign data protection laws and regulations (e.g., laws and regulations that address data privacy and data security including, without limitation, health data). These new or proposed laws and regulations are subject to differing interpretations and may be inconsistent among jurisdictions, and guidance on implementation and compliance practices are often updated or otherwise revised, which adds to the complexity of processing personal data. These and other requirements could require us or our partners to incur additional costs to achieve compliance, limit our competitiveness, necessitate the acceptance of more onerous obligations in our contracts, restrict our ability to use, store, transfer, and process data, impact our or our partners' ability to process or use data in order to support the provision of our products or services, affect our or our partners' ability to offer our products and services in certain locations, or cause regulators to reject, limit or disrupt our clinical trial activities.

In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure, and protection of health-related and other personal information could apply to our operations or the operations of our partners. In addition, we may receive unintended health information in error from third parties (including research institutions from which we may obtain clinical trial data) that are subject to privacy and security requirements under the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH). Depending on the facts and circumstances, we could be subject to criminal penalties, including if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

International data protection laws, including, without limitation, the EU General Data Protection Regulation (GDPR) that took effect in May 2018, and member state data protection legislation, may also apply to health-related and other personal information obtained outside of the United States. These laws impose strict obligations on the ability to process health-related and other personal information of data subjects in the EU, including in relation to use, collection, analysis, and transfer of such personal information. These laws include several requirements relating to obtaining the consent of the individuals to whom the personal data relates, limitations on data processing, establishing a legal basis for processing, notification of data processing obligations or security incidents to appropriate data protection authorities or data subjects, the security and confidentiality of the personal data and various rights that data subjects may exercise.

The GDPR generally restrict the transfer of personal information from Europe, including the European Economic Area, United Kingdom and Switzerland, to the United States and most other countries unless the parties to the transfer have implemented specific safeguards to protect the transferred personal information. One of the primary safeguards allowing United States companies to import personal information from Europe has been certification to the EU-U.S. Privacy Shield and Swiss-U.S. Privacy Shield frameworks administered by the United States Department of Commerce. However, the Court of Justice of the EU recently invalidated the EU-U.S. Privacy Shield. The same decision also raised questions about whether one of the primary alternatives to the EU-U.S. Privacy Shield, namely, the European Commission's Standard Contractual Clauses, can lawfully be used for personal information transfers from Europe to the United States or most other countries. At present, there are few, if any, viable alternatives to the EU-U.S. Privacy Shield and the Standard Contractual Clauses. Although we rely primarily on individuals' explicit consent to transfer their personal information from Europe to the United States and other countries, in certain cases we have relied or may rely on the Standard Contractual Clauses. Authorities in the United Kingdom and Switzerland, whose data protection laws are similar to those of the EU, may similarly invalidate use of the EU-U.S. Privacy Shield and Swiss-U.S. Privacy Shield, respectively, as mechanisms for lawful personal information transfers from those countries to the United States. As such, if we are unable to rely on explicit consent to transfer individuals' personal information from Europe, which can be revoked, or implement another valid compliance solution, we will face increased exposure to substantial fines under European data protection laws as well as injunctions against processing personal information from Europe. Inability to import personal information from the European Economic Area, United Kingdom or Switzerland may also restrict our clinical trial activities in Europe; limit our ability to collaborate with CROs, service providers, contractors and other companies subject to European data protection laws; and require us to increase our data processing capabilities in Europe at significant expense. Additionally, other countries outside of Europe have enacted or are considering enacting similar cross-border data transfer restrictions and laws requiring local data residency, which could increase the cost and complexity of delivering our services and operating our business.

Under the GDPR, regulators may impose substantial fines and penalties for non-compliance. Companies that violate the GDPR can face fines of up to the greater of 20 million Euros or 4% of their worldwide annual turnover (revenue). The GDPR has increased our responsibility and liability in relation to personal data that we process, requiring us to put in place additional mechanisms to ensure compliance with the GDPR and other EU and international data protection rules.

In addition, in June 2018, California enacted the California Consumer Privacy Act (CCPA), which went into effect on January 1, 2020. The CCPA gives California residents expanded rights to access and require deletion of their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that may increase data breach litigation. Although the CCPA includes exemptions for certain clinical trials data, and HIPAA protected health information, the law may increase our compliance costs and potential liability with respect to other personal information we collect about California residents. The CCPA has prompted a number of proposals for new federal and state privacy legislation that, if passed, could increase our potential liability, increase our compliance costs and adversely affect our business.

Failure to comply with U.S. and international data protection laws and regulations could result in government enforcement actions (which could include civil or criminal penalties, fines or sanctions), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, patients about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations related to security or privacy, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business. Compliance with data protection laws may be time-consuming, require additional resources and could result in increased expenses, reduce overall demand for our products and services and make it more difficult to meet expectations of or commitments to customers or partners.

Any of these matters could materially adversely affect our business, financial condition, or operational results.

Failure in our information technology and storage systems or our security measures, including without limitation, data breaches, or inadequacy of our business continuity and disaster recovery plans and procedures, could significantly disrupt the operation of our business.

Our ability to execute our business plan and maintain operations depends on the continued and uninterrupted performance of our information technology (IT) systems, and the availability of data related to our products, services and operations. IT systems and data are vulnerable to risks and damages from a variety of sources, including catastrophe or natural disaster, telecommunications or network failures, malicious human acts, breaches of security, cyber-attacks, loss of power or other natural or man-made events. Moreover, despite network security and back-up measures, some of our and our vendors' servers are potentially vulnerable to physical or electronic break-ins, computer viruses and similar disruptive problems. If our business continuity and disaster recovery plans and procedures were disrupted, inadequate or unsuccessful in the event of a problem, we could experience a material adverse interruption of our operations.

Specifically, data security breaches, whether inadvertent or intentional, by employees or others, may expose proprietary information, trade secrets, personal information, clinical trial data or other sensitive data to unauthorized persons, impact the integrity, availability or confidentiality of our IT systems or data (including, but not limited to, data loss), or disrupt or interrupt our IT systems or operations. Our partners and vendors face similar risks and any security breach of their systems could adversely affect our security posture. Malicious attacks by third parties are of ever-increasing sophistication and can be made by groups and individuals with a wide range of motives (including, but not limited to, industrial espionage) and expertise, including organized criminal groups, "hacktivists," nation states and others. Foreign, federal, and state laws or regulations allows for the imposition of civil liability, fines and/or corrective action on entities that improperly use or disclose the personal information of individuals, including through a data security breach. Accordingly, data security breaches experienced by us, our collaborators or contractors could lead to significant fines, required corrective action, loss of trade secrets or other intellectual property, or could lead to the public exposure of personally identifiable information (including sensitive personal information) of our employees, collaborators, clinical trial patients, and others. A data security breach or privacy violation that leads to disclosure or modification of or prevents access to personal information, including personally identifiable information, patient information or protected health information, could result in civil liability, harm our reputation, compel us to comply with federal and/or state breach notification laws, subject us to mandatory corrective action, require us to verify the correctness of database contents and otherwise subject us to liability under laws and regulations that protect personal data, resulting in increased costs or loss of revenue. If we are unable to prevent data security breaches or privacy violations, respond appropriately or implement satisfactory remedial measures, our operations could be disrupted, and we may suffer civil liability to our customers or individuals, loss of reputation, financial loss and other regulatory penalties because of lost or misappropriated information, including sensitive patient data. In addition, these breaches and other inappropriate access events can be difficult to detect, and any delay in identifying and responding to them may lead to increased harm of the type described above. Moreover, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information, trade secrets or other intellectual property. While we have implemented security measures designed to protect our data security and information technology systems, no set of security measures is infallible, and these measures may not prevent such events.

Despite precautionary measures to prevent anticipated and unanticipated problems, including data breaches, there can be no assurance that our efforts to protect our data and information technology systems will prevent breakdowns or breaches in our systems (or that of our third-party providers). Such events could affect our IT systems, sustained or repeated system failures that interrupt our ability to generate, use and maintain data or our IT systems could adversely affect our ability to operate our business and result in increased costs or loss of revenue, other financial and reputational harm to us, theft of trade secrets and other proprietary information, legal claims or proceedings, liability under laws that protect the privacy of personal information and regulatory penalties.

We may not be successful in any efforts to identify, license, discover, develop or commercialize additional product candidates.

Although a substantial amount of our effort will focus on the continued clinical testing, potential approval, and commercialization of our existing product candidates, the success of our business is also expected to depend in part upon our ability to identify, license, discover, develop, or commercialize additional product candidates. Research programs to identify new product candidates require substantial technical, financial, and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. Our research programs or licensing efforts may fail to yield additional product candidates for clinical development and commercialization for a number of reasons, including but not limited to the following:

- our research or business development methodology or search criteria and process may be unsuccessful in identifying potential product candidates;
- we may not be able or willing to assemble sufficient resources to acquire or discover additional product candidates;
- our product candidates may not succeed in preclinical or clinical testing;
- our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval;
- competitors may develop alternatives that render our product candidates obsolete or less attractive;
- product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the market for a product candidate may change during our program so that such a product may become unreasonable to continue to develop;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, or we may not be able to identify, license, discover, develop, or commercialize additional product candidates, which would have a material adverse effect on our business and could potentially cause us to cease operations.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (ACA) was passed, which substantially changed the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. There remains judicial and Congressional challenges to numerous provisions of the ACA, as well as efforts by the Trump administration to repeal or replace certain aspects of the ACA. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the ACA such as removing penalties, starting January 1, 2019, for not complying with the ACA's individual mandate to carry health insurance and delaying the implementation of certain ACA-mandated fees. On December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Cuts and Jobs Act of 2017. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. It is unclear how this decision, future decisions, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA and our business. In addition, since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of any certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Further, the Bipartisan Budget Act of 2018, among other things, amends the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." In addition, the 2020 federal spending package permanently eliminates, effective January 1, 2020, the ACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Also, there has been heightened governmental scrutiny recently over pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between

pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. For example, the Trump administration released a “Blueprint” to lower drug prices and reduce out-of-pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out-of-pocket costs of drug products paid by consumers. In addition, the Trump administration’s budget proposal for fiscal year 2021 includes a \$135.0 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs. The Department of Health and Human Services, or HHS, has solicited feedback on some of these measures and, at the same time, has implemented others under its existing authority. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage plans the option to use step therapy for Part B drugs beginning January 1, 2020. This final rule codified CMS’s policy change that was effective January 1, 2019. While some of these and other measures may require additional authorization to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Any repeal and replacement legislation may have the effect of limiting the amounts that government agencies will pay for healthcare products and services. Policy changes, including potential modification or repeal of all or parts of the ACA or the implementation of new health care legislation, could result in significant changes to the health care system, which may prevent us from being able to generate revenue, attain profitability or commercialize our drugs. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand or lower pricing for our product candidates, or additional pricing pressures.

In the United States, the EU and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. For example, in the United States, there have been several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the EU will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

We may be subject, directly or indirectly, to foreign, federal and state healthcare fraud and abuse laws, false claims laws, and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties, sanctions or other liability.

Our operations may be subject to various foreign, federal and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, physician sunshine laws, the GDPR and other regulations. These laws may impact, among other things, our proposed sales, marketing, and education programs. In addition, we may be subject to patient privacy regulation by foreign, federal, and state governments in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;
- HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- HIPAA and its implementing regulations impose certain requirements on certain covered entity healthcare providers, health plans, and healthcare clearinghouse as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, relating to the privacy, security, and transmission of individually identifiable health information;

- The Physician Payments Sunshine Act, which requires manufacturers of drugs, devices, biologics, and medical supplies to report annually to the U.S. Department of Health and Human Services information related to payments and other transfers of value to physicians, as defined by such law, and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members and applicable group purchasing organizations;
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws that may apply to items or services reimbursed by any third-party payors, including commercial insurers, state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; state and local laws requiring the registration of pharmaceutical sales representatives; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts; and
- the GDPR and other EU member state data protection legislation, which require data controllers and processors, to adopt administrative, physical and technical safeguards to protect personal data, including health-related data, including mandatory contractual terms with third-party providers, requirements for establishing an appropriate legal basis for processing personal data, transparency requirements related to communications with data subjects regarding the processing their personal data, standards for obtaining consent from individuals to process their personal data, notification requirements to individuals about the processing of their personal data, an individual data rights regime, mandatory data breach notifications, limitations on the retention of personal data, increased requirements pertaining to health data, as well as strict rules and restrictions on the transfer of personal data outside of the EU, including to the United States.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has strengthened these laws.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply, we may be subject to penalties, including significant civil, criminal and administrative penalties, damages, disgorgement, fines, sanctions, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

The withdrawal of the United Kingdom from the EU, commonly referred to as “Brexit,” may adversely impact our ability to obtain regulatory approvals of our product candidates in the EU, result in restrictions or imposition of taxes and duties for importing our product candidates into the EU, and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the EU.

On June 23, 2016, the U.K. held a referendum in which a majority of the eligible members of the electorate voted for the U.K. to leave the EU. The U.K.'s withdrawal from the EU is commonly referred to as Brexit. The U.K. and the EU agreed to a withdrawal agreement (the ***Withdrawal Agreement***) pursuant to which the U.K. formally left the EU on January 31, 2020. Under the Withdrawal Agreement, the U.K. is subject to a transition period until December 31, 2020 (the ***Transition Period***), during which EU rules will continue to apply. Due to the current COVID-19 global pandemic, negotiations between the U.K. and the EU, scheduled for March are either being postponed or occurring in a reduced forum via video conference. There is, therefore, an increased likelihood that the Transition Period may need to be extended beyond December 31, 2020 (although it remains the position of the U.K. government that it will not be extended).

Since a significant proportion of the regulatory framework in the U.K. applicable to our business and our product candidates is derived from EU directives and regulations, Brexit, following the Transition Period, could materially impact the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the U.K. or the EU. For example, as a result of the uncertainty surrounding Brexit, the European Medicines Agency (EMA) relocated to Amsterdam from London. Following the Transition Period, the U.K. will no longer be covered by the centralized procedures for obtaining EU-wide marketing authorization from the EMA and, unless a specific agreement is entered into, a separate process for authorization of drug products, including our product candidates, will be required in the U.K., the potential process for which is currently unclear. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the U.K. or the EU and restrict our ability to generate revenue and achieve and sustain profitability. In addition, we may be required to pay taxes or duties or be subjected to other hurdles in connection with the importation of our product candidates into the EU, or we may incur expenses in establishing a manufacturing facility in the EU in order to circumvent such hurdles. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the U.K. or the EU for our product candidates, or incur significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between the impacted nations and the U.K. It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the EU.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraudulent conduct or other illegal activity by our employees, independent contractors, principal investigators, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, promotion, sales, marketing and certain business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of patient recruitment or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of business conduct and ethics applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use or misuse of our product candidates harm patients or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted, and we could be subject to costly and damaging product liability claims.

The use or misuse of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation;
- initiation of investigations by regulators;
- withdrawal of clinical trial participants;
- costs due to related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates;
- product recalls, withdrawals or labeling, marketing or promotional restrictions; and
- decreased demand for our product candidates, if approved for commercial sale.

We believe our current product liability insurance coverage is appropriate in light of our clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to increase our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. A successful product liability claims or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Patients with the diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may or may not be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use, and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our licensors and our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently, and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. We do not currently carry biological or hazardous waste insurance coverage.

We are currently conducting and will continue to conduct clinical trials in foreign countries, which could expose us to risks that could have a material adverse effect on the success of our business and the delivery of clinical trial data.

We have conducted in the past and are currently conducting clinical trials in the United States, Canada, Australia, Turkey, Germany, Pakistan, New Zealand, Mongolia, Spain, France, Bulgaria, Romania, Taiwan, Sweden, Italy, Belgium, Switzerland, United Kingdom, Greece, Moldova, Ukraine, Russia, and Israel, and accordingly, we are subject to risks associated with doing business globally, including commercial, political, and financial risks. Emerging regions, such as Eastern Europe, Latin America, Asia, and Africa, as well as more developed markets, such as the United Kingdom, France, Germany, and Australia, provide clinical study opportunities for us. In addition, we are subject to potential disruption caused by military conflicts; potentially unstable governments or legal systems; civil or political upheaval or unrest; local labor policies and conditions; possible expropriation, nationalization, or confiscation of assets; problems with repatriation of foreign earnings; economic or trade sanctions; closure of markets to imports; anti-American sentiment; terrorism or other types of violence in or outside the United States; health pandemics; and a significant reduction in global travel. For example, both Turkey and Pakistan are key regions for clinical activity relating to Hepatitis Delta Virus, and further outbreaks of violence and political instability in the region could disrupt our clinical operations or otherwise limit our ability to access or conduct clinical studies in those regions. Certain countries have closed their borders due to COVID-19 preventing activation of clinical sites. Our success will depend, in part, on our ability to overcome the challenges we encounter with respect to these risks and other factors affecting U.S. companies with global operations. If our global clinical trials were to experience significant disruption due to these risks or for other reasons, it could have a material adverse effect on our financial results.

We or the third parties upon whom we depend may be adversely affected by earthquakes, natural epidemics or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Earthquakes, health epidemics or other natural disasters could severely disrupt our operations and have a material adverse effect on our business. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, damaged critical infrastructure, such as the manufacturing facilities of our third-party CMOs, or otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. For example, in December 2019, an outbreak of a novel strain of coronavirus (COVID-19) originated in Wuhan, China. Since certain starting materials of certain of our products obtained from third-party chemical suppliers are manufactured in China and Japan, an outbreak of communicable diseases in the region, or the perception that such an outbreak could occur, and the measures taken by the governments of countries affected, could adversely affect our business, financial condition or results of operations by limiting our ability to manufacture product within or outside for example China, Japan, Italy, Canada, and the United States, forcing temporary closure of facilities that we rely upon or increasing the costs associated with obtaining starting materials and then clinical supplies of our product candidates. The extent to which the coronavirus impacts our results will depend on future developments, which are highly uncertain and cannot be accurately predicted, including new information which may emerge concerning the severity of the coronavirus and the actions to contain the coronavirus or treat its impact, among others. In addition, our corporate headquarters is located in the San Francisco Bay Area, which has in the past experienced severe earthquakes and other natural disasters and is currently experiencing an outbreak of COVID-19. We do not carry earthquake insurance. We have limited disaster recovery and business continuity plans in place currently and our business would be impaired in the event of a serious disaster or similar event. We may incur substantial expenses to develop and implement any disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Our business is currently adversely affected by and could be materially adversely affected in the future by the effects of disease outbreaks, epidemics and pandemics including the evolving effects of the COVID-19 outbreak. We have a significant number of clinical trial sites in countries that have been directly affected by COVID-19. We depend on manufacturing operations for various stages of our supply chain in countries that have been directly affected by COVID-19. COVID-19 continues to adversely affect our business and could materially and adversely affect our operations and those of our manufacturers and other third parties with whom we conduct business.

Our business has been adversely affected by COVID-19 and could be materially and adversely impacted by COVID-19 or other health epidemics in regions where we have significant manufacturing facilities, concentrations of clinical trial sites or other business operations.

The COVID-19 pandemic has presented a substantial public health and economic challenge around the world and is affecting employees, patients, community and business operations, as well as the U.S. economy and financial markets. The effects of the shelter-in-place order and our work-from-home policies may negatively impact productivity, disrupt our business and delay our clinical programs and timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. These and similar, and perhaps more severe, disruptions in our operations could negatively impact our business, operating results and financial condition.

Additionally, some of our suppliers of certain materials used in the production of our drug products are located in China, Japan, Canada, Italy and the United States. While many of these materials may be obtained by more than one supplier, including suppliers outside of China, Japan, Canada, Italy and the United States, port closures and other restrictions resulting from the coronavirus outbreak in the region may disrupt our supply chain or limit our ability to obtain sufficient materials for our drug products.

In addition, our clinical trials have been and may continue to be affected by the COVID-19 pandemic. Site initiation and patient enrollment has been delayed, due to prioritization of hospital resources toward the COVID-19 pandemic, travel restrictions imposed by governments, and the inability to access sites for initiation and monitoring. In our D-LIVR trial, the COVID-19 pandemic has delayed enrollment in our global clinical trial, some patients may not be able to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services, we may be unable to obtain blood samples for testing, and we may not be able to provide study drug to patients.

Further, as a result of the COVID-19 pandemic, the extent and length of which is uncertain, we may be required to develop and implement additional clinical study policies and procedures designed to help protect study participants from the COVID-19 virus, which may include using telemedicine visits, remote monitoring of patients and clinical sites, and measures to ensure that data from clinical studies that may be disrupted as result of the pandemic are collected pursuant to the study protocol and consistent with good clinical practices (GCPs), with any material protocol deviation reviewed and approved by the site Institutional Review Board (IRB). Missed scheduled patient appointments, any interruption in study drug supply, or other consequences that may result in incomplete data being generated during a study as a result of the pandemic must be adequately documented and justified. For example, on March 18, 2020, the FDA issued guidance on conducting clinical trials during the pandemic, which describes a number of considerations for sponsors of clinical trials impacted by the pandemic, including the requirement to include in the clinical study report (or as a separate document) contingency measures implemented to manage the study, any disruption of the study as a result of the COVID-19 pandemic; a list of all study participants by unique subject identifier and by investigational site that were affected by the COVID-19 pandemic, and a description of how the individual's participation was altered; and analyses and corresponding discussions that address the impact of implemented contingency measures (e.g., participant discontinuation from investigational product and/or study, alternative procedures used to collect critical safety and/or efficacy data) on the safety and efficacy results reported for the study.

Further, the FDA may continue to suspend or delay certain foreign inspections, and the EMA may also, and if there continues to be a suspension or delay in inspections, our product application reviews and potential approvals could be impacted or delayed.

While we expect the COVID-19 pandemic to continue to adversely affect our business operations, the extent of the impact on our clinical development and regulatory efforts and the value of and market for our common stock will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat COVID-19. In addition, to the extent the evolving effects of the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described elsewhere in this "Risk Factors" section.

Risks Related to Ownership of our Common Stock

The market price of our common stock has been and may continue to be highly volatile, and you may not be able to resell some or all of your shares at a desired market price.

The market price of our common stock has been and is likely to continue to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- results or delays in preclinical studies or clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- unanticipated serious safety concerns related to the use of any of our product candidates;
- reports of adverse events in other gene therapy products or clinical trials of such products;
- inability to obtain additional funding;
- any delay in filing an IND, NDA, or MAA for any of our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND or NDA;
- our ability to obtain regulatory approvals for our product candidates, and delays or failures to obtain such approvals;
- failure of any of our product candidates, if approved, to achieve commercial success;
- failure to obtain Orphan Drug designation;
- failure to maintain our existing third-party license and supply agreements;
- failure by our licensors to prosecute, maintain, or enforce our intellectual property rights;
- changes in laws or regulations applicable to our product candidates;
- any inability to obtain adequate supply of our product candidates or the inability to do so at acceptable prices;
- adverse regulatory authority decisions;
- introduction of new products, services, or technologies by our competitors;
- failure to meet or exceed financial and development projections we may provide to the public;
- failure to meet or exceed the financial and development projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators, and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- announcements by commercial partners or competitors of new commercial products, clinical progress or the lack thereof, significant contracts, commercial relationships or capital commitments;
- adverse publicity relating to the hepatitis market generally, including with respect to other products and potential products in such markets;
- the introduction of technological innovations or new therapies that compete with potential products of ours;

- changes in the structure of health care payment systems; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies, including very recently in connection with the ongoing COVID-19 pandemic, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. These broad market and industry factors, including potentially worsening economic conditions and other adverse effects or developments relating to the ongoing COVID-19 pandemic, may also adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

Effective December 31, 2019, we ceased to be an “emerging growth company,” and the reduced reporting requirements applicable to “emerging growth companies” no longer apply, which has increased our costs as a result of being a public company and places additional demands on management.

Effective December 31, 2019, we ceased to be classified as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act (the JOBS Act). We are now considered an Accelerated Filer and a Smaller Reporting Company, which will require accelerated deadlines of periodic reports. In March 2020, the definition of an Accelerated Filer was amended to exclude Smaller Reporting Companies from the requirements of the Sarbanes-Oxley Act Section 404(b).

We have previously taken advantage of the JOBS Act's reduced disclosure requirements applicable to “emerging growth companies” regarding executive compensation and exemptions from the requirements of holding advisory say-on-pay votes on executive compensation. Since we are no longer classified as an “emerging growth company,” we are no longer eligible for such reduced disclosure requirements and exemptions and as such, we are required to hold a say-on-pay vote and a say-on-frequency vote at our 2019 annual meeting of stockholders. As a result, we expect that because we are no longer classified as an “emerging growth company,” we will require additional attention from management with respect to our disclosures and will incur increased costs, which could include higher legal fees, accounting fees, consultant fees and fees associated with investor relations activities, among others.

We will incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.

We have incurred and will continue to incur significant legal, accounting and other expenses associated with public company reporting requirements. We also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as rules implemented by the SEC and The Nasdaq Stock Market LLC. These rules and regulations impose significant legal and financial compliance costs and make some activities more time-consuming and costly. For example, our management team consists of certain executive officers who have not previously managed and operated a public company. These executive officers and other personnel will need to devote substantial time to gaining expertise regarding operations as a public company and compliance with applicable laws and regulations. In addition, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as executive officers, which may adversely affect investor confidence and could cause our business or stock price to suffer.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management.

Provisions in our certificate of incorporation and bylaws may delay or prevent an acquisition or a change in management. These provisions include a classified board of directors, a prohibition on actions by written consent of our stockholders and the ability of the board of directors to issue preferred stock without stockholder approval. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which prohibits stockholders owning in excess of 15% of our voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

We do not anticipate that we will pay any cash dividends in the foreseeable future.

We expect to retain our future earnings to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders, if any, for the foreseeable future.

Future sales of shares by existing stockholders could cause our stock price to decline.

If existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after legal restrictions on resale lapse, the trading price of our common stock could decline. Certain of our existing stockholders, including RA Capital, Vivo Ventures Fund VI, L.P. and Adage Capital Partners, and their respective affiliated entities, own substantial ownership interest in our common stock and any decision to sell a significant number of shares may negatively impact the price of our common stock.

The ownership of our common stock is highly concentrated, and it may prevent stockholders from influencing significant corporate decisions and may result in conflicts of interest that could cause our stock price to decline.

Our executive officers, directors and 5% stockholders and their affiliates beneficially own or control a significant portion of the outstanding shares of our common stock. Accordingly, these executive officers, directors, 5% stockholders and their affiliates, acting as a group, have substantial influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transactions. These stockholders may also delay or prevent a change of control of us, even if such a change of control would benefit our other stockholders. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flows, financial condition or results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business and financial condition. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act (Tax Act), enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act repealed or modified in future legislation. For example, the CARES Act modified certain provisions of the Tax Act. In addition, it is uncertain if and to what extent various states will conform to the Tax Act or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Act or future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Our net operating loss carryforwards and certain other tax attributes are now subject to limitations.

Our federal and state net operating loss (NOL) carry-forwards will begin to expire, if not utilized, beginning in 2030 for federal income tax purposes and 2028 for California state income tax purposes. These NOL carry-forwards could expire unused and be unavailable to offset future income tax liabilities. While the Tax Act allows for federal net operating losses incurred in 2019 and in future years to be carried forward indefinitely, the deductibility of such federal net operating losses incurred in 2019 and in future years will be limited. Moreover, if a corporation undergoes an ownership change within the meaning of Section 382 of the Code (Section 382) the corporation's NOL carryforwards and certain other tax attributes arising from before the ownership change are subject to limitations on use after the "ownership change." In general, an ownership change occurs if there is a cumulative change in the corporation's equity ownership by certain stockholders that exceeds fifty percentage points over a rolling three-year period. Similar rules may apply under state tax laws. Our merger with Celladon resulted in such an ownership change and, accordingly, Celladon's NOL carryforwards and certain other tax attributes will be subject to further limitations on their use. In addition, we assessed whether Eiger had an ownership change, as defined by Section 382 of the Code, as a result of the Merger and other stock issuances that occurred from our formation through August 5, 2019. Based upon this assessment, we have experienced ownership changes on April 20, 2016 and October 18, 2018. Due to these ownership changes, reductions were made to our NOL and tax credit carryforwards under these rules. Additional ownership changes in the future could result in additional limitations on our net operating loss and tax credit carryforwards. Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our NOL carryforwards and other tax attributes, which could have a material adverse effect on cash flow and results of operations. A full valuation allowance has been provided for the entire amount of our remaining net operating losses.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

<u>Exhibit Number</u>	<u>Description of Document</u>
3.1	<u>Amended and Restated Certificate of Incorporation of Celladon Corporation (incorporated by reference to Exhibit 3.1 to the Current Report on Form 8-K of Celladon Corporation, filed with the SEC on February 10, 2014).</u>
3.2	<u>Amended and Restated Bylaws of Celladon Corporation (incorporated by reference to Exhibit 3.2 to the Current Report on Form 8-K, filed with the SEC on February 10, 2014).</u>
3.3	<u>Certificate of Amendment of Amended and Restated Certificate of Incorporation of Celladon Corporation (incorporated by reference to Annex D to the Registration Statement on Form S-4, as amended (File No. 333-208521), originally filed with the SEC on December 14, 2015).</u>
3.4	<u>Certificate of Amendment of Amended and Restated Certificate of Incorporation of Celladon Corporation (incorporated by reference to Annex E to the Registration Statement on Form S-4, as amended (File No. 333-208521), originally filed with the SEC on December 14, 2015).</u>
31.1**	<u>Certification of Principal Executive Officer required by Rule 13a-14(a) or Rule 15d-14(a).</u>
31.2**	<u>Certification of Principal Financial Officer required by Rule 13a-14(a) or Rule 15d-14(a).</u>
32.1+	<u>Certifications of Principal Executive Officer and Principal Financial Officer required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350).</u>
101.INS**	Inline XBRL Instance Document- the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH**	Inline XBRL Taxonomy Extension Schema Document
101.CAL**	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF**	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB**	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE**	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	The cover page from the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, has been formatted in Inline XBRL.

+ This certification accompanies the Quarterly Report pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not be deemed "filed" by the Registrant for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.

** Filed herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Quarterly Report on Form 10-Q to be signed on its behalf by the undersigned thereunto duly authorized.

Date: November 5, 2020

Eiger BioPharmaceuticals, Inc.

By: /s/ David A. Cory
David A. Cory
Director, President and Chief Executive Officer
(Principal Executive Officer)

Date: November 5, 2020

Eiger BioPharmaceuticals, Inc.

By: /s/ Sriram Ryali
Sriram Ryali
Chief Financial Officer
(Principal Financial Officer)

**Certification of President and Chief Executive Officer
Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002**

I, David A. Cory, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Eiger BioPharmaceuticals, 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 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 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 functions):
 - 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.

Date: November 5, 2020

/s/ David A. Cory

David A. Cory
Chief Executive Officer

**Certification of Chief Financial Officer
Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002**

I, Sriram Ryali, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Eiger BioPharmaceuticals, 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 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 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 functions):
 - 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.

Date: November 5, 2020

/s/ Sriram Ryali
Sriram Ryali
Chief Financial Officer

CERTIFICATION

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the “Exchange Act”) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), David A. Cory, Chief Executive Officer of Eiger BioPharmaceuticals, Inc. (the “Company”), and Sriram Ryali, Chief Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company’s Quarterly Report on Form 10-Q for the period ended September 30, 2020, to which this Certification is attached as Exhibit 32.1 (the “Periodic Report”), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and
2. The information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 5, 2020

/s/ David A. Cory

David A. Cory
Chief Executive Officer

/s/ Sriram Ryali

Sriram Ryali
Chief Financial Officer

“This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Eiger BioPharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.”