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

Form 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2022

OR

o 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	**33-0971591**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**
2155 Park Boulevard Palo Alto , CA	**94306**
(Address of Principal Executive Offices)	**(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 x No o

Indicate by check mark whether the registrant has submitted electronically, every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§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 x No o

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

Large accelerated filer	o	Accelerated filer	o
Non-accelerated filer	x	Smaller reporting company	x
Emerging growth company	o		

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

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

As of November 1, 2022, the number of outstanding shares of the registrant's common stock, par value $0.001 per share, was 44,074,284.

Our independent registered public accounting firm is KPMG LLP, San Francisco, CA, Auditor ID: 185

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, 2022 (Unaudited)	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 26,308	$ 22,221
Short-term debt securities	94,736	66,594
Accounts receivable, net	2,458	2,576
Inventories	2,817	2,612
Prepaid expenses and other current assets	15,970	9,361
Total current assets	142,289	103,364
Long-term debt securities	—	17,262
Property and equipment, net	511	613
Operating lease right-of-use assets	246	653
Other assets	698	4,510
Total assets	$ 143,744	$ 126,402
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 10,497	$ 7,765
Accrued liabilities	15,345	13,699
Current portion of operating lease liabilities	277	628
Debt, current portion	—	7,809
Total current liabilities	26,119	29,901
Debt, net of current portion	39,315	23,986
Operating lease liabilities	2	116
Total liabilities	65,436	54,003
Stockholders' equity:		
Common stock	44	35
Additional paid-in capital	490,939	412,930
Accumulated other comprehensive loss	(620)	(149)
Accumulated deficit	(412,055)	(340,417)
Total stockholders' equity	78,308	72,399
Total liabilities and stockholders' equity	$ 143,744	$ 126,402

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,	
	2022	2021	2022	2021
Product revenue, net	$ 4,024	$ 3,039	$ 10,038	$ 8,782
Other revenue	—	—	750	—
Total revenue	4,024	3,039	10,788	8,782
Costs and operating expenses:				
Cost of sales	1,231	318	1,492	641
Research and development	22,198	18,106	56,761	46,250
Selling, general and administrative	6,964	6,466	20,804	17,916
Total costs and operating expenses	30,393	24,890	79,057	64,807
Loss from operations	(26,369)	(21,851)	(68,269)	(56,025)
Interest expense	(1,092)	(894)	(2,912)	(2,659)
Interest income	347	35	613	119
Other (expense) income, net	3	503	(1,044)	46,462
Loss before provision for income taxes	(27,111)	(22,207)	(71,612)	(12,103)
Provision for income taxes	—	16	26	46
Net loss	$ (27,111)	$ (22,223)	$ (71,638)	$ (12,149)
Net loss per common share:				
Basic and diluted	$ (0.62)	$ (0.65)	$ (1.76)	$ (0.36)
Weighted-average common shares outstanding:				
Basic and diluted	44,010,553	33,946,559	40,806,581	33,922,080

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,	
	2022	2021	2022	2021
Net loss	$ (27,111)	$ (22,223)	$ (71,638)	$ (12,149)
Other comprehensive loss:				
Unrealized gain (loss) on available-for-sale debt securities, net	141	7	(471)	9
Comprehensive loss	$ (26,970)	$ (22,216)	$ (72,109)	$ (12,140)

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, 2021	34,568,821	$ 35	$ 412,930	$ (149)	$ (340,417)	$ 72,399
Issuance of common stock upon offering at-the-market, net of $1,288 of commissions	5,841,786	6	45,604	—	—	45,610
Issuance of common stock upon exercise of stock options	15,995	—	144	—	—	144
Vesting of common stock issued under Product Development Agreement	—	—	19	—	—	19
Issuance of common stock upon ESPP purchase	18,130	—	64	—	—	64
Issuance of common stock upon release of restricted stock units	85,106	—	—	—	—	—
Stock-based compensation expense	—	—	2,047	—	—	2,047
Unrealized loss on available-for-sale debt securities, net	—	—	—	(373)	—	(373)
Net loss	—	—	—	—	(22,643)	(22,643)
Balance at March 31, 2022	40,529,838	41	460,808	(522)	(363,060)	97,267
Issuance of common stock upon offering at-the-market, net of $716 of commissions and issuance costs	2,686,288	2	20,562	—	—	20,564
Issuance of common stock to lender	749,053	1	4,999	—	—	5,000
Issuance of common stock upon exercise of stock options	1,604	—	9	—	—	9
Stock-based compensation expense	—	—	2,208	—	—	2,208
Unrealized loss on available-for-sale debt securities, net	—	—	—	(239)	—	(239)
Net loss	—	—	—	—	(21,884)	(21,884)
Balance at June 30, 2022	43,966,783	44	488,586	(761)	(384,944)	102,925
Issuance of common stock upon exercise of stock options	3,512	—	27	—	—	27
Issuance of common stock upon ESPP purchase	29,985	—	104	—	—	104
Issuance of common stock upon release of restricted stock units	47,748	—	—	—	—	—
Stock-based compensation expense	—	—	2,222	—	—	2,222
Unrealized gain on available-for-sale debt securities, net	—	—	—	141	—	141
Net loss	—	—	—	—	(27,111)	(27,111)
Balance at September 30, 2022	44,048,028	$ 44	$ 490,939	$ (620)	$ (412,055)	$ 78,308

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, 2020	33,878,486	$ 34	$ 401,509	$ (8)	$ (306,500)	$ 95,035
Issuance of common stock upon exercise of stock options	19,150	—	166	—	—	166
Vesting of common stock issued under Product Development Agreement	—	—	53	—	—	53
Issuance of common stock upon ESPP purchase	19,928	—	136	—	—	136
Issuance of common stock upon release of restricted stock units	33,750	—	—	—	—	—
Stock-based compensation expense	—	—	1,549	—	—	1,549
Unrealized gain on available-for-sale debt securities, net	—	—	—	3	—	3
Net income	—	—	—	—	29,248	29,248
Balance at March 31, 2021	33,951,314	34	403,413	(5)	(277,252)	126,190
Vesting of common stock issued under Product Development Agreement	—	—	52	—	—	52
Stock-based compensation expense	—	—	2,058	—	—	2,058
Unrealized loss on available-for-sale debt securities, net	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(19,174)	(19,174)
Balance at June 30, 2021	33,951,314	34	405,523	(6)	(296,426)	109,125
Vesting of common stock issued under Product Development Agreement	—	—	62	—	—	62
Issuance of common stock upon release of restricted stock units	6,795	—	43	—	—	43
Issuance of common stock upon ESPP purchase	17,691	—	121	—	—	121
Stock-based compensation expense	—	—	2,330	—	—	2,330
Unrealized gain on available-for-sale debt securities, net	—	—	—	7	—	7
Net income	—	—	—	—	(22,223)	(22,223)
Balance at September 30, 2021	33,975,800	$ 34	$ 408,079	$ 1	$ (318,649)	$ 89,465

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,	
	2022	2021
Operating activities		
Net loss	$ (71,638)	$ (12,149)
Adjustments to reconcile net loss to net cash used in operating activities:		
Gain from sale of priority review voucher	—	(46,493)
Income related to asset purchase agreement	—	(281)
Depreciation and amortization	219	203
Inventory write down	1,043	—
Amortization of debt securities premiums and discounts	698	658
Loss on extinguishment of debt	1,144	—
Non-cash interest expense	867	577
Amortization of operating lease right-of-use assets	407	399
Common stock issued under Product Development Agreement	19	167
Stock-based compensation	6,477	5,937
Change in operating assets and liabilities:		
Accounts receivable	118	(2,762)
Inventories	(892)	(2,528)
Prepaid expenses and other current assets	(2,181)	493
Other assets	(616)	(837)
Accounts payable	2,722	2,218
Accrued liabilities	1,315	257
Operating lease liabilities	(465)	(436)
Net cash used in operating activities	(60,763)	(54,577)
Investing activities		
Purchase of debt securities available-for-sale	(55,538)	(71,447)
Proceeds from maturities of debt securities available-for-sale	43,489	99,630
Proceeds related to asset purchase agreement	—	281
Proceeds from sale of priority review voucher	—	95,000
Payments related to priority review voucher	—	(48,507)
Purchase of property and equipment	(116)	(92)
Net cash (used in) provided by investing activities	(12,165)	74,865
Financing activities		
Issuance of common stock upon offering at-the-market, net of commissions	66,402	—
Proceeds from issuance of common stock to lender	5,000	—
Proceeds from debt	39,840	—
Repayment of debt	(33,277)	—
Proceeds from issuance of common stock upon stock option exercises	180	209
Proceeds from issuance of common stock upon ESPP purchase	168	277
Payment of debt issuance costs	(1,054)	(175)
Common stock offering costs	(244)	(208)
Net cash provided by financing activities	77,015	103
Net increase in cash and cash equivalents	4,087	20,391
Cash and cash equivalents at beginning of period	22,221	28,864
Cash and cash equivalents at end of period	$ 26,308	$ 49,255
Supplemental disclosure of cash flow information:		
Interest paid	$ 2,038	$ 2,082
Income taxes paid	$ 43	$ —

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 commercial-stage biopharmaceutical company focused on the development of innovative therapies for hepatitis delta virus (HDV), the most severe form of viral hepatitis, and other serious diseases. All five of the Company's rare disease programs have been granted Breakthrough Therapy Designation by the U.S. Food and Drug Administration (FDA).

The Eiger HDV platform includes two first-in-class therapies in Phase 3 that target critical host processes involved in viral replication. Lonafarnib is a first-in-class, oral farnesylation inhibitor and peginterferon lambda is a first-in-class, type III, interferon.

The FDA approved the Company's first commercial product, Zokinvy® (lonafarnib), to reduce risk of mortality of Hutchinson-Gilford progeria syndrome (HGPS) and for treatment of processing-deficient progeroid laminopathies (PL), collectively known as progeria, with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, on November 20, 2020. The Company announced that the European Commission approved its Marketing Authorization Application (MAA) for Zokinvy, under exceptional circumstances procedure on July 20, 2022.

The Company is also developing avexitide, a well-characterized peptide, as a treatment for congenital hyperinsulinism (HI), an ultra-rare pediatric metabolic disorder, and post-bariatric hypoglycemia (PBH), a debilitating and potentially life-threatening condition. There are currently no approved therapies for these disorders.

The Company's principal operations are based in Palo Alto, California, with a subsidiary in Ireland. The Company operates in one segment.

Liquidity

As of September 30, 2022, the Company had $121.0 million of cash, cash equivalents and short-term securities, comprised of $26.3 million of cash and cash equivalents and $94.7 million of short-term debt securities available-for-sale. The Company had an accumulated deficit of $412.1 million and negative cash flows from operating activities as of September 30, 2022. As the Company continues to incur losses, its transition to profitability will depend on the successful development, approval, and commercialization of product candidates and on the achievement of sufficient revenues to support its cost structure. The Company may never achieve profitability, and until it does, the Company will need to continue to raise additional capital.

Management believes that the currently available resources will be sufficient to fund its planned operations for at least the next 12 months following the issuance date of these condensed consolidated financial statements. However, if the Company's anticipated operating results are not achieved in future periods, the Company believes that planned expenditures may need to be reduced or it would be required to raise funding in order to fund the operations. Additionally, the Company's ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and 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 follow the requirements of the Securities and Exchange Commission (SEC) for annual reporting. All intercompany balances and transactions have been eliminated in consolidation.

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 revenue and expenses during the reporting period. On an ongoing basis, the Company evaluates its estimates. 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. Long-term securities consist of debt securities classified as available-for-sale and have maturities greater than 365 days from the date of acquisition. The Company's debt securities consist of available-for-sale securities that are classified as Level 2 because their value is based on valuations using significant inputs derived from, or corroborated by, observable market data. Unrealized gains and losses on available-for-sale debt securities are excluded from earnings and are reported as a component of accumulated other comprehensive (loss) income. 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) income, net on the accompanying unaudited condensed consolidated statements of operations.

Accounts Receivable

Accounts receivable represent amounts billed to the Company's customers, net of an allowance for doubtful accounts. Trade accounts receivable are recorded at invoiced amounts and do not bear interest. The expectation of collectability is based on a review of credit profiles of customers, contractual terms and conditions, current economic trends, and historical payment experience. The Company regularly reviews the adequacy of the allowance for doubtful accounts by considering the age of each outstanding invoice and the collection history of each customer to determine the appropriate amount of allowance for doubtful accounts.

The Company had no allowance as of September 30, 2022 and December 31, 2021. The Company had no bad debt expense for the three and nine months ended September 30, 2022 and 2021.

Inventories

Inventories are stated at the lower of cost, determined based on actual costs, or estimated net realizable value, on a first-in, first-out basis. Inventories consist of raw materials, work-in-process, and finished goods.

Prior to regulatory approval of the Company's product candidates, expenses incurred to manufacture drug products are recorded as research and development expense. The Company begins capitalizing these expenses as inventory upon regulatory approval.

The Company periodically assesses the recoverability of its inventory and reduces the carrying value of the inventory when items are determined to be obsolete, defective or in excess of forecasted sales requirements. Inventory write-downs for excess, defective, and obsolete inventory are recorded as a cost of sales.

The Company wrote-down $1.0 million of inventories for the nine months ended September 30, 2022. There were no write-downs in prior periods.

Revenue Recognition

The Company recognizes revenue upon transfer of control of promised products to customers in an amount that reflects the consideration it expects to receive in exchange for those products. To determine revenue recognition for contracts with customers, the Company performs the following five-step approach: (i) identify the contract, or contracts, with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when, or as, the performance obligation is satisfied. The five-step model is only applied to contracts when it is probable that the Company will collect substantially all of the consideration it is entitled to in exchange for the goods transferred to a customer.

Product Revenue

The Company's product revenue consists of sales of Zokinvy, which received FDA approval in November 2020 and was launched commercially in the United States in January 2021. Prior to 2021, the Company had no product revenue. In the United States, the Company sells Zokinvy to a single specialty pharmacy provider that subsequently dispenses the product directly to patients. The Company discloses revenue on a total basis without further disaggregation. Additionally, the Company does not have any contract assets or liabilities, other than accounts receivable, related to its product revenue.

In June 2021, the French National Agency for Medicines and Health Products Safety (ANSM) granted Zokinvy (lonafarnib) a Temporary Authorizations for Use (Autorisation Temporaire d'Utilisation or ATU) for an early access program for a term of one year. The Company has received an extension of the ATU program and expects the program to continue until commercial reimbursement of Zokinvy is approved in France. In the context of this program, the Company sells product to a distributor who in turn ships product to pharmacies after receiving requests from physicians for patients in France. In November 2021, the Company began distributing and recognizing revenue from sales of Zokinvy (lonafarnib) through a reimbursed early access program in France. There was no revenue from sale of product under the ATU program for the nine months ended September 30, 2022.

The Company recognizes product revenue when a customer obtains control of its product, which occurs at a point in time, typically upon delivery to a customer as the delivery of the product is the Company's only performance obligation. Shipping and handling activities are fulfillment activities rather than a separate performance obligation and are recorded in cost of sales.

Product revenue is recorded at the net sales price (transaction price), which includes estimates of variable consideration resulting from rebates, prompt payment discounts, co-payment assistance, and returns. Amounts related to such items are estimated at contract inception and updated at the end of each reporting period as additional information becomes available. The amount of variable consideration may be constrained and is included in the transaction price only to the extent it is probable that a significant reversal of cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is resolved. Product revenue is recorded after considering the impact of the following variable consideration amounts along with the constraint at the time of revenue recognition:

Rebates: The Company's product is subject to government mandated rebates for Medicaid Drug Rebate Program, Medicare Part D Prescription Drug Benefit Program, and other government health care programs in the United States. Rebate amounts are based upon contractual agreements or legal requirements with public sector benefit providers. The Company uses the expected-value method for estimating these rebates based on statutory discount rates and expected utilization. The expected utilization of rebates is estimated based on expected coverage of identified patients. Estimates for these rebates are adjusted quarterly to reflect the most recent information. The Company records an accrued liability for unpaid rebates related to products for which control has been transferred to a customer.

Prompt payment discounts: The Company provides a discount to a customer if it pays for purchases within 30 days. The Company expects that its customers will earn prompt payment discounts and uses the most likely amount method for estimating such discounts. As a result, when revenues are recognized, the Company deducts the full amount of the prompt payment discounts from total product revenues and records these discounts as a reduction of accounts receivable.

Co-payment assistance: The Company provides co-payment assistance to patients who have commercial insurance and meet certain eligibility requirements. The Company uses the expected-value method for estimating co-payment assistance based on estimates of program redemption using data provided by third-party administrators. Estimates for the co-payment assistance are adjusted quarterly to reflect actual experience. The Company records an accrued liability for unredeemed co-payment assistance related to products for which control has been transferred to a customer.

Product returns: The Company offers limited product return rights and generally allows for the return of product that is damaged or defective, or within a few months prior to and up to a few months after the product expiration date. The Company considers several factors in the estimation of potential product returns, including expiration dates of the product shipped, the limited product return rights, third-party data in monitoring channel inventory levels, shelf life of the product, and other relevant factors.

Other Revenue

The Company's other revenue consists of milestone payments from the Marketing and Distribution Agreement (MDA) with AnGes, Inc., which was executed in May 2022. The agreement provides AnGes with a right to use the Company's

intellectual property (IP) and seek regulatory approval for and commercialization of Zokinvy in Japan. The Company will receive additional payments upon achievement of certain regulatory milestones.

Cost of Sales

Cost of sales consists primarily of direct and indirect costs related to the manufacturing of Zokinvy for commercial sale, including third-party manufacturing costs, packaging services, freight, storage costs, and write down of inventories.

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 accompanying unaudited condensed consolidated balance sheets and within research and development expenses in the accompanying 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.

Recent Accounting Pronouncements

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 unaudited condensed consolidated financial statements.

In March 2020, the FASB issued ASU No. 2020-04, *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. In October 2021, the Company amended its Oxford Loan to replace its floating interest rate with the LIBOR replacement rate (see Note 7). The Company adopted this standard when LIBOR was about to be discontinued and the adoption did not have a material impact on its unaudited condensed consolidated financial statements.

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, 2022 and December 31, 2021, the carrying amount of accounts receivable, prepaid expenses and other current assets, accounts payable and accrued liabilities approximated their estimated 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, 2022 and December 31, 2021.

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, 2022			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	$ 13,121	$ —	$ —	$ 13,121
Corporate debt securities	—	40,088	—	40,088
Commercial paper	—	10,874	—	10,874
U.S. government bonds	—	43,774	—	43,774
Total	$ 13,121	$ 94,736	$ —	$ 107,857

	December 31, 2021			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	$ 13,520	$ —	$ —	$ 13,520
Corporate debt securities	—	41,511	—	41,511
U.S. government bonds	—	42,345	—	42,345
Total	$ 13,520	$ 83,856	$ —	$ 97,376

There were no financial liabilities as of September 30, 2022 and December 31, 2021.

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, 2022			
	Amortized cost	Unrealized gain	Unrealized loss	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 13,121	$ —	$ —	$ 13,121
Total cash equivalents	$ 13,121	$ —	$ —	$ 13,121
Debt securities:				
Corporate debt securities	$ 40,398	$ —	$ (310)	$ 40,088
Commercial paper	10,874	—	—	10,874
U.S. government bonds	44,084	—	(310)	43,774
Total debt securities	$ 95,356	$ —	$ (620)	$ 94,736
Classified as:				
Cash equivalents				$ 13,121
Short-term debt securities				94,736
				$ 107,857

	December 31, 2021			
	Amortized cost	Unrealized gain	Unrealized loss	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 13,520	$ —	$ —	$ 13,520
Total cash equivalents	$ 13,520	$ —	$ —	$ 13,520
Debt securities:				
Corporate debt securities	$ 41,576	$ —	$ (65)	$ 41,511
U.S. government bonds	42,429	—	(84)	42,345
Total debt securities	$ 84,005	$ —	$ (149)	$ 83,856
Classified as:				
Cash equivalents				$ 13,520
Short-term debt securities				66,594
Long-term debt securities				17,262
				$ 97,376

The Company periodically reviews the available-for-sale securities 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, 2022, the Company did not recognize any other-than-temporary impairment losses. The unrealized losses of $0.6 million as of September 30, 2022 include debt securities with unrealized losses of $0.1 million that have been in the loss position for more than 12 months. However, the Company is planning to hold these securities until maturity and expects to recover the amortized cost basis.

4. Balance Sheet Components

Inventories

Inventories consist of the following (in thousands):

	September 30, 2022	December 31, 2021
Raw materials	$ 1,704	$ 1,056
Work-in-progress	1,009	1,468
Finished goods	104	88
Total inventories	$ 2,817	$ 2,612

Prepaid Expenses and Other Current Assets

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

	September 30, 2022	December 31, 2021
Prepaid contract manufacturing costs	$ 5,083	$ 3,695
Short term deposits	4,630	54
Prepaid research costs	3,103	3,253
Prepaid insurance	876	631
Prepaid marketing	484	469
Other	1,794	1,259
Total prepaid expenses and other current assets	$ 15,970	$ 9,361

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	September 30, 2022	December 31, 2021
Contract manufacturing costs	$ 4,453	$ 3,288
Contract research costs	4,098	4,760
Compensation and related benefits	3,530	3,131
Product revenue reserves	2,035	1,846
Other	1,229	674
Total accrued liabilities	$ 15,345	$ 13,699

5. Bristol-Meyers Squibb License Agreement

On April 20, 2016, the Company and Bristol-Myers Squibb Company (BMS) entered into a License Agreement (the BMS License Agreement) and a Common Stock Purchase Agreement (the BMS Purchase Agreement).

Under the BMS License Agreement, BMS granted the Company an exclusive, worldwide, license to research, develop, manufacture, and sell products containing PEG-interferon Lambda-1a (peginterferon lambda or the Licensed Product) for all therapeutic and diagnostic uses in humans and animals. The Company is responsible for the development and commercialization of the Licensed Product at its sole cost and expense. The Company paid BMS $2.0 million and issued 157,587 shares of its common stock at an aggregate fair value of $3.2 million in April 2016. The BMS License Agreement also includes development and regulatory milestone payments totaling $61.0 million and commercial sales milestones of up to $128.0 million. The Company is obligated to pay BMS annual net sales royalties in the range of mid-single to mid-teens, depending on net sales levels. In fourth quarter of 2020, the Company recorded in research and development expense a $3.0 million milestone, triggered on successful demonstration of proof of concept, as defined by the BMS License Agreement, in a Phase 2 clinical trial. In March 2022, the Company recorded a $5.0 million milestone expense in research

and development, which was related to the initiation of a Phase 3 clinical trial, as defined under the BMS License Agreement.

6. Asset Purchase Agreement

On November 20, 2020, Eiger entered into an asset purchase agreement (the APA) with AbbVie, Inc. (AbbVie) to sell its rare pediatric disease priority review voucher (the PRV), which was awarded on November 20, 2020 upon FDA approval of Zokinvy. The APA contains customary representations, warranties, covenants, and indemnification provisions subject to certain limitations.

In consideration for the PRV, AbbVie agreed to pay the Company $95.0 million. The transaction closed in January 2021. Such consideration was shared with the Progeria Research Foundation (PRF) in accordance with the terms of the PRF Collaboration and Supply Agreement, pursuant to which the Company and PRF will equally share any net proceeds from the sale of a priority review voucher that the Company may receive as the sponsor of a rare pediatric disease product application. The Company retained $46.5 million of proceeds from the sale of the PRV, net of related payments, and recorded the amount in other (expense) income, net in the unaudited condensed consolidated statement of operations for the nine months ended September 30, 2021.

7. Debt

Innovatus Term Loan

On June 1, 2022 (Closing Date), the Company entered into a term loan and security agreement (Innovatus Loan) with Innovatus Life Sciences Lending Fund I, LP (Innovatus), providing for up to $75.0 million funded in three tranches with a maturity date of August 31, 2027. The floating per annum interest rate of the Innovatus Loan is equal to the sum of (a) the greater of (i) the Prime Rate published in the Money Rates section of the Wall Street Journal (or any successor thereto) and (ii) 3.5%, plus (b) 3.75%; provided that, at the election of the Borrower, up to 2.25% of such rate shall be payable in-kind until the third anniversary of the closing date. The Company is required to make monthly interest-only payments through July 1, 2027, after which the Company is required to make monthly amortizing payments, with the remaining balance of the principal plus accrued and unpaid interest due at maturity. 2.25% of the interest is payable in-kind for the first three years of the term by increasing the principal balance. Prepayments of the loan, in whole or in part, will be subject to an early prepayment fee which declines each year until the third anniversary date of the Closing Date, after which no prepayment fee is required. The Company is also required to pay an exit fee upon any payment or prepayment equal to 6.5% of the aggregate principal amount of the tranches funded under the Innovatus Loan. The Innovatus Loan contains customary representations, warranties, events of default and covenants of the Company and its subsidiaries, including a requirement to maintain a cash balance of not less than 5% of the aggregate principal amount of funded and outstanding loan terms at all times. The Innovatus Loan is secured by 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 Company was funded $40.0 million in June 2022 on the Closing Date under Tranche A. The remaining $35.0 million is divided into two tranches (Tranche B and Tranche C). The $17.5 million under each of Tranche B and Tranche C will be available for a period commencing on the later of (a) the first date that the Company achieves certain development and regulatory milestones applicable to each Tranche and (b) November 1, 2022. Both Tranche B and Tranche C draw periods end on the earlier of (a) June 30, 2024 or (b) an event of default.

The Company identified a number of embedded derivatives that require bifurcation from the Innovatus Loan. These embedded features include mandatory prepayment upon an event of default or change in control and contingent rate increases. However, the fair value of these embedded features was deemed to be immaterial on the date of issuance. At each subsequent reporting period, the Company will reassess the fair value of the embedded features and will record a liability if the fair value of the features becomes material.

In connection with the issuance of the Innovatus Loan, the Company recorded a debt discount of $0.2 million and capitalized debt issuance costs of $1.0 million. The discount and issuance costs will be amortized over the life of the loan. Interest expense for the Innovatus Loan for the three and nine months ended September 30, 2022 was $1.1 million and $1.4 million, respectively, and is inclusive of non-cash amortization of the debt discount and debt issuance costs and accretion of final payment. The carrying value of the Innovatus Loan at September 30, 2022 was $39.3 million. The

carrying amount of the Innovatus Loan approximates fair value given its recent issuance. The effective interest rate for the Innovatus Loan was 11.59% as of September 30, 2022.

Additionally, in connection with entering into the Innovatus Loan, the Company entered into a Stock Purchase Agreement with Innovatus for the sale of common stock with an aggregate value of $5.0 million. On June 1, 2022, the Company issued 749,053 shares of common stock to Innovatus at a per share purchase price of $6.6751, the preceding five-day volume weighted average price per share.

A portion of the loan proceeds were used to repay in full the approximately $33.5 million of aggregate principal amount, unpaid interest, and exit fees in connection with loans outstanding owed to Oxford Finance LLC by the Company.

Oxford Term Loan

In December 2016, the Company entered into an aggregate $25.0 million loan with Oxford Finance LLC (the Oxford Loan) with a maturity date of 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.

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) was available to the Company provided that certain milestones are achieved by February 2021. The Company did not draw down the Amended Tranche B.

On February 23, 2021, the Company entered into the fifth amendment to the Oxford Loan. The amendment extended the interest only period by 17 months until September 1, 2022, followed by 19 equal monthly payments of principal and interest. The Company paid an amendment fee of $0.2 million to the lender on the effective date of the fifth amendment, which was recorded as an additional debt discount and was being amortized over the remaining term of the Amended Oxford Loan. Interest expense for the Oxford Loan for the nine months ended September 30, 2022 was $1.5 million.

On October 6, 2021, the Company entered into the sixth amendment to the Oxford Loan, which amended the interest to be the LIBOR replacement rate which is the sum of the alternate benchmark rate and the LIBOR replacement spread.

At the time of final payment, the Company was required to pay an exit fee of 7.50% of the original principal balance of borrowed funds, or $2.3 million. In addition, the Company was 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.

On June 1, 2022, upon entering into the Innovatus Loan, the Company repaid the Oxford Loan, including (i) the $30.0 million outstanding principal balances, (ii) $0.2 million in accrued and unpaid interest, and (iii) other final payments consisting of $3.3 million, for a total payment of $33.5 million. The Company recorded a loss of $1.1 million on early extinguishment of the debt related to the unamortized debt premium, discount, and cost of issuance, which was recognized as a component of other (expense) income, net in the condensed consolidated statement of operations and other comprehensive loss.

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

	September 30, 2022	December 31, 2021
Face value of debt	$ 40,302	$ 30,000
Exit fee	2,600	3,277
Unamortized debt discount associated with exit fee, debt issuance costs and loan origination fees	(3,587)	(1,482)
Total debt, net	39,315	31,795
Less: current portion of debt	—	(7,809)
Debt, net	$ 39,315	$ 23,986

8. Stock-Based Compensation

During the second quarter of 2021, the Company approved the 2021 Inducement Plan to reserve 850,000 shares of its common stock to be used exclusively for grants of awards to individuals that were not previously employees or directors of the Company as a material inducement to such individuals' entry into employment with the Company within the meaning of Rule 5635(c)(4) of the Nasdaq Listing Rules. As of September 30, 2022, there were 380,000 shares remaining available to be issued under the 2021 Inducement Plan.

The following table summarizes stock option activity, including restricted stock units (RSUs) and performance stock units (PSUs) available for grant activity, under the Company's stock-based compensation plans during the nine months ended September 30, 2022 (in thousands, except option and share data):

	Shares Available for Grants	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2021	1,327,645	5,262,185	$ 10.24	7.25	$ 530
Additional options authorized	1,728,441				
Granted	(1,987,100)	1,987,100	$ 5.80		
Restricted stock units granted	(298,150)	—			
Performance stock units granted	(30,000)	—			
Exercised		(21,111)	$ 8.50		
Forfeited	351,495	(351,495)	$ 9.74		
Restricted stock units forfeited	64,940	—			
Outstanding as of September 30, 2022	1,157,271	6,876,679	$ 8.99	7.22	$ 5,877
Vested and exercisable as of September 30, 2022		3,724,985	$ 10.54	5.78	$ 2,148

During the three and nine months ended September 30, 2022, the weighted-average grant date fair value of options granted were $5.46 and $3.68 per share, respectively. During the three and nine months ended September 30, 2021, the weighted-average grant date fair value of options granted were $4.65 and $6.78 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,	
	2022	2021	2022	2021
Expected term (in years)	5.77-6.08	5.77-6.08	5.27-6.08	5.27-6.08
Volatility	71.44%-73.08%	70.72%-71.62%	68.71%-73.08%	70.72%-100.87%
Risk free interest rate	2.70%-4.02%	0.89%-1.15%	1.76%-4.02%	0.62%-1.16%
Dividend yield	—	—	—	—

Restricted Stock Units and Performance Stock Units

In the first quarter of 2020, the Company revised its non-employee director compensation policy to approve the granting of RSUs in accordance with the Restated 2013 Equity Incentive Plan (the Restated 2013 Plan). Each eligible director who has served for at least six months during the prior calendar year and continues to serve as a non-employee member of the board of Directors (the Board) is granted RSUs. Each eligible director who has served on the Board for less than six months during the prior calendar year and who 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. The RSUs granted to employees will vest annually on the one-year, two-year, and three-year anniversaries of the grant date. The fair value of all RSUs is 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.

The PSUs are also available for grant pursuant to the Restated 2013 Plan. Each PSU, which is a stock award, is earned through the achievement of performance-based metrics over a defined performance period. The length of the defined performance period, the performance-based metric to be achieved during the defined performance period, and the measure of whether and to what degree such performance-based metric has been achieved will be conclusively determined by the compensation committee of the Company's Board, in its sole discretion. The estimated fair value of equity awards that contain performance conditions is expensed over the term of the award once the Company has determined that it is probable that performance conditions will be satisfied. During the three and nine months ended September 30, 2021, the Company granted 240,000 PSUs with a weighted-average grant date fair value of $8.15 per share. During the nine months ended September 30, 2022, the Company granted 30,000 PSUs, with a weighted-average grant date fair value of $6.87 per share. There were no PSUs granted during the three months ended September 30, 2022. As of September 30, 2022, no PSUs have vested as the performance-based metrics of the PSUs have not yet been achieved.

There were no RSUs granted during the three months ended September 30, 2022. During the three months ended September 30, 2021, the Company granted 168,000 RSUs, with a weighted-average grant date fair value of $8.15 per share. During the nine months ended September 30, 2022 and 2021, the Company granted 298,150 and 371,500 RSUs, respectively, with a weighted-average grant date fair value of $5.22 and $9.16 per share, respectively.

In relation to the RSUs granted, the Company recognized $0.5 million and $0.4 million in stock-based compensation expense for the three months ended September 30, 2022 and 2021, respectively, and $1.4 million and $0.7 million in stock-based compensation expense for the nine months ended September 30, 2022 and 2021, respectively, which were included in selling, general and administrative expenses. As of September 30, 2022, the total unrecognized compensation expense related to unvested RSUs and PSUs was $4.1 million, which the Company expects to recognize over an estimated weighted-average period of 2.30 years.

The following table summarizes RSU and PSU activity and weighted average grant date fair value for the nine months ended September 30, 2022:

	Shares	Weighted-Average Grant Date Fair Value
Unvested shares as of December 31, 2021	623,000	$ 8.63
Granted	328,150	$ 5.37
Vested	(132,854)	$ 9.40
Forfeited	(64,940)	$ 7.95
Unvested shares as of September 30, 2022	753,356	$ 7.13

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,	
	2022	2021	2022	2021
Research and development	$ 856	$ 686	$ 2,301	$ 1,627
Selling, general and administrative	1,366	1,644	4,176	4,310
Total	$ 2,222	$ 2,330	$ 6,477	$ 5,937

As of September 30, 2022, the total unrecognized compensation expense related to unvested options was $13.7 million, which the Company expects to recognize over an estimated weighted average period of 2.8 years.

9. Income Taxes

The Company’s provision for income taxes was approximately $0 and $26,000 for the three and nine months ended September 30, 2022, respectively, with an effective tax rate of (0.04)% for the nine months ended September 30, 2022. The Company's provision for income taxes was approximately $16,000 and $46,000 for the three and nine months ended September 30, 2021, with an effective tax rate of (0.3)% for the nine months ended September 30, 2021. The effective tax rate in each period differs from the U.S. statutory tax rate primarily due to the valuation allowances on the Company’s deferred tax assets as it is more likely than not that some or all of the Company’s deferred tax assets will not be realized. The tax expense recorded for the nine months ended September 30, 2022 relates to state taxes.

10. 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. The lease commenced on March 1, 2018 and expires in February 2023. The lease has a three-year renewal option prior to expiration; and the Company is evaluating its plans and is not yet 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 additional operating leases that are included in its lease accounting but are not considered significant for disclosure.

The maturities of the Company's operating lease liabilities as of September 30, 2022 were as follows (in thousands):

Undiscounted lease payments	September 30, 2022
Remaining in 2022	$ 167
2023	115
2024	1
2025	1
Total undiscounted payments	284
Less: imputed interest	5
Present value of future lease payments	279
Less: current portion of operating lease liabilities	277
Operating lease liabilities	$ 2

Rent expense recognized for the Company's operating leases was $0.1 million for the three months ended September 30, 2022 and 2021, and $0.4 million for the nine months ended September 30, 2022 and 2021. 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 for the three months ended September 30, 2022 and 2021 and $0.1 million for the nine months ended September 30, 2022 and 2021.

The operating cash outflows for the operating lease liabilities were $0.5 million for the nine months ended September 30, 2022 and 2021. As of September 30, 2022 and December 31, 2021, the weighted-average remaining lease terms were 0.5 years and 1.2 years, and weighted-average discount rates were 9.15% and 9.15%, respectively.

11. 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 the three months ended September 30, 2022 and 2021, and nine months ended September 30, 2022 and 2021, diluted net loss per share is the same as basic net loss per share as the inclusion of all potential common shares outstanding would have been anti-dilutive. Dilutive potential common stock equivalents include the assumed exercise, vesting and issuance of employee stock awards using the treasury stock method.

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,	
	2022	2021	2022	2021
Options to purchase common stock	6,876,679	5,378,092	6,876,679	5,378,092
Restricted stock units (unvested)	753,356	627,088	753,356	627,088
ESPP	85,110	36,431	85,110	37,409
Total	7,715,145	6,041,611	7,715,145	6,042,589

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 condensed consolidated financial statements and related notes thereto for the year ended December 31, 2021, included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on March 10, 2022. 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 commercial-stage biopharmaceutical company focused on the development of innovative therapies for hepatitis delta virus (HDV) and other serious diseases. All five of our rare disease programs have FDA Breakthrough Therapy Designation.

Our HDV platform includes two first-in-class therapies in Phase 3 that target critical host processes involved in viral replication. Lonafarnib is a first-in-class, oral farnesylation inhibitor and peginterferon lambda is a first-in-class, type III, interferon.

D-LIVR is the pivotal Phase 3 study of lonafarnib boosted with ritonavir, alone or in combination with peginterferon alfa-2a, for HDV. The study completed enrollment of 407 patients, and we expect topline data by the end of 2022. LIMT-2 is the pivotal Phase 3 study of peginterferon lambda for HDV.

We are also developing avexitide, a first in class, well-characterized GLP-1 antagonist, as a targeted treatment for two metabolic diseases with high unmet medical needs and no approved therapies: congenital hyperinsulinism (HI) and post-bariatric hypoglycemia (PBH). Avexitide has completed Phase 2 for both indications, and we initiated Phase 3 for HI in 2022.

The FDA approved our first commercial product, Zokinvy (lonafarnib), to reduce risk of mortality of Hutchinson-Gilford progeria syndrome (HGPS) and for treatment of processing-deficient progeroid laminopathies (PL) with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, on November 20, 2020. Collectively known as progeria. these are ultra-rare and rapidly fatal genetic conditions of accelerated aging in children. On July 20, 2022, we announced that the European Commission (EC) granted Eiger a centralized marketing authorization (MA) under the exceptional circumstances procedure for Zokinvy for the treatment of HGPS and PL,ultra-rare and rapidly fatal genetic conditions of accelerated aging in children. The MA is

subject to the EMA’s continued review on an annual basis of new efficacy and safety information which may become available. The EC's centralized MA is valid in all 27 EU member states plus Iceland, Liechtenstein, and Norway. Regulatory review is ongoing by the UK's Medicine and Healthcare products Regulatory Agency (MHRA) as part of the UK's European Commission Decision Reliance Procedure.

We commercially launched Zokinvy in the U.S. in January 2021 and started to record product revenue in first quarter 2021. We have historically incurred operating losses in each year since inception and we expect to incur losses for the foreseeable future. We had a net loss of $27.1 million and $22.2 million for the three months ended September 30, 2022 and 2021, respectively. We had a net loss of $71.6 million and $12.1 million for the nine months ended September 30, 2022 and 2021, respectively. As of September 30, 2022, we had an accumulated deficit of $412.1 million. Substantially all of our operating losses have resulted from expenses incurred in connection with our research and development programs and from selling, 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, which will require the addition of new personnel and upgrades to our information technology systems. 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 additional regulatory approvals.

Recent Developments

Update on Plans for Emergency Use Authorization (EUA) Application Following U.S. Food and Drug Administration (FDA) Feedback

On October 5, 2022, we announced that, following feedback from FDA, we will not submit an EUA application of peginterferon lambda for the treatment of patients with mild-to-moderate COVID-19.

Following our press release on September 6, 2022, in which we updated on the status of our planned EUA based on recent communications with the FDA, we submitted a pre-EUA meeting request to FDA as well as additional morbidity and mortality outcomes data and analyses from the investigator-sponsored TOGETHER study. This included further statistical modeling and efficacy analyses of the study's primary and secondary endpoints and long-term follow-up data that we believe continues to support the topline outcomes reported in March. In response, FDA denied the request for a pre-EUA meeting. Citing its concerns with the conduct of the TOGETHER study, FDA concluded that any authorization request based on these data is unlikely to meet the statutory criteria for issuance of an EUA in the current context of the pandemic.

FDA suggested that, given peginterferon lambda's mechanism of action and the ongoing need for improved COVID-19 therapeutics, we should consider requesting an end-of-Phase 2 meeting to discuss a company-sponsored pivotal trial that could support an eventual Biologics License Application (BLA), We are evaluating next steps for this program in the U.S., as well as ex-U.S. emergency use authorization pathways, and strategic options for the continued development of peginterferon lambda for COVID-19 and other respiratory viral infections.

Financial Operations Overview

Product Revenue, Net

Our product revenue, net consists of sales of Zokinvy® (lonafarnib) for progeria and processing deficient progeroid laminopathies in the United States and under a reimbursed early access program, or cohort ATU program, in France.

Other Revenue

Other revenue consists of milestone payments from the MDA with AnGes, Inc., which was executed in May 2022.

Cost of Sales

Cost of sales consists primarily of direct and indirect costs related to the manufacturing of Zokinvy for commercial sale, including third-party manufacturing costs, third party logistics costs, write down of inventories, and other period costs.

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, 2022 and 2021 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Product candidates:				
Lonafarnib	$ 6,877	$ 9,276	$ 19,385	$ 24,609
Peginterferon lambda	6,964	5,379	17,359	12,292
Avexitide	3,254	507	6,976	1,303
Internal research and development costs	5,103	2,944	13,041	8,046
Total research and development expense	$ 22,198	$ 18,106	$ 56,761	$ 46,250

We expect research and development expenses will continue to be significant and may 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, in the future we may 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.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist of personnel costs, allocated expenses, expenses for outside professional services, including legal, audit, accounting services, insurance costs and costs associated with being a public company, and commercial related expenses. 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. We expect our selling, general and administrative expenses to increase in the future due to sales and marketing activities from the commercialization of our product candidates.

Interest Expense

Interest expense consists of interest on our long-term borrowings.

Interest Income

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

Other (expense) income, net

Other (expense) income, net primarily consists of the loss on extinguishment of debt recorded during the nine months ended September 30, 2022.

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 revenues, 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 revenues and expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. There were no material changes to our critical accounting policies as disclosed in our 2021 Annual Report on Form 10-K filed with SEC on March 10, 2022.

Results of Operations

Comparison of the Three Months Ended September 30, 2022 and 2021

The following table summarizes results of operations for the three months ended September 30, 2022 and 2021 (in thousands):

	Three Months Ended September 30,		$ Change	% Change
	2022	2021		
Product revenue, net	$ 4,024	$ 3,039	$ 985	32 %
Total revenue	4,024	3,039	985	32 %
Costs and operating expenses:				
Cost of sales	1,231	318	913	287 %
Research and development	22,198	18,106	4,092	23 %
Selling, general and administrative	6,964	6,466	498	8 %
Total costs and operating expenses	30,393	24,890	5,503	22 %
Loss from operations	(26,369)	(21,851)	(4,518)	21 %
Interest expense	(1,092)	(894)	(198)	22 %
Interest income	347	35	312	891 %
Other (expense) income , net	3	503	(500)	(99)%
Loss before provision for income taxes	(27,111)	(22,207)	(4,904)	22 %
Provision for income taxes	—	16	(16)	(100)%
Net loss	$ (27,111)	$ (22,223)	$ (4,888)	22 %

Product revenue, net

Product revenue, net increased by $1.0 million to $4.0 million for the three months ended September 30, 2022, from $3.0 million for the same period in 2021. The increase was primarily due to an increase in units shipped during the quarter.

Cost of sales

Cost of sales increased by $0.9 million to $1.2 million for the three months ended September 30, 2022, from $0.3 million for the same period in 2021. The increase was primarily due to a non-conforming batch of inventory that was written off during the three months ended September 30, 2022.

Research and development expenses

Research and development expenses increased by $4.1 million to $22.2 million for the three months ended September 30, 2022, from $18.1 million for the same period in 2021. The increase was primarily due to a $2.0 million increase in headcount related expenses, including stock-based compensation expense, a $2.0 million increase contract manufacturing expenditures related to timing of production of clinical materials across programs, a $0.3 million increase in clinical trial related expenses for avexitide and peginterferon lambda, and a $0.1 million increase in activities related to participation in scientific conferences. These increases were partially offset by a $0.5 million decrease in outside services, including consulting and advisory services, primarily associated with peginterferon lambda.

Selling, general and administrative expenses

Selling, general and administrative expenses increased by $0.5 million to $7.0 million for the three months ended September 30, 2022, from $6.5 million for the same period in 2021. The increase was primarily due to a $0.4 million increase in outside services, including consulting, advisory and accounting services and a $0.1 million increase in other operating expenses, all to support company growth.

Interest expenses

Interest expense increased by $0.2 million compared to the same period in 2021. The increase was primarily due to an increase in outstanding principle balance related to the Innovatus loan.

Interest income

Interest income increased by $0.3 million compared to the same period in 2021. The increase was primarily due to an increase in the balance of money market funds and available-for-sale securities along with an increase in rates.

Other (expense) income, net

Other (expense) income, net decreased by $0.5 million compared to the same period in 2021. The decrease was primarily due to the proceeds from a corporate insurance settlement during the three months ended September 30, 2021.

Provision for income taxes

Provision for income taxes decreased by $16,000 compared to the same period in 2021. The change was primarily due to a decrease in state taxes.

Comparison of the Nine Months Ended September 30, 2022 and 2021

The following table summarizes results of operations for the nine months ended September 30, 2022 and 2021 (in thousands):

	Nine Months Ended September 30,		$ Change	% Change
	2022	2021		
Product revenue, net	$ 10,038	$ 8,782	$ 1,256	14 %
Other revenue	750	—	750	*
Total revenue	10,788	8,782	2,006	23 %
Costs and operating expenses:				
Cost of sales	1,492	641	851	133 %
Research and development	56,761	46,250	10,511	23 %
Selling, general and administrative	20,804	17,916	2,888	16 %
Total costs and operating expenses	79,057	64,807	14,250	22 %
Loss from operations	(68,269)	(56,025)	(12,244)	22 %
Interest expense	(2,912)	(2,659)	(253)	10 %
Interest income	613	119	494	415 %
Other (expense) income, net	(1,044)	46,462	(47,506)	(102)%
Loss before provision for income taxes	(71,612)	(12,103)	(59,509)	492 %
Provision for income taxes	26	46	(20)	(43)%
Net loss	$ (71,638)	$ (12,149)	$ (59,489)	490 %

*Percentage not meaningful or not material.

Product revenue, net

Product revenue, net increased by $1.2 million to $10.0 million for the nine months ended September 30, 2022, from $8.8 million for the same period of 2021. The increase was primarily due to an increase in units shipped during the year.

Other Revenue

Other revenue of $0.8 million for the nine months ended September 30, 2022 reflects the upfront payment received from AnGes, Inc. under the terms of the MDA which was executed in May 2022.

Cost of sales

Cost of sales increased by $0.9 million to $1.5 million for the nine months ended September 30, 2022, from $0.6 million for the same period in 2021. The increase was primarily due to a non-conforming batch of inventory that was written off during the nine months ended September 30, 2022.

Research and development expenses

Research and development expenses increased by $10.5 million to $56.8 million for the nine months ended September 30, 2022, from $46.3 million for the same period in 2021. The increase was primarily due to a $5.0 million milestone related to the Phase 3 LIMT-2 study of peginterferon lambda for HDV under the BMS License Agreement, which occurred in March 2022, a $4.7 million increase in headcount related expenses, including stock-based compensation expense, a $2.3 million increase in clinical trial related expenses for peginterferon lambda and avexitide, and a $0.2 million increase in activities related to participation in scientific conferences. These increases were partially offset by a $1.0 million decrease in contract manufacturing expenditures related to timing of production of clinical materials across programs and a $0.7 million decrease in outside services, including, consulting and advisory services, primarily associated with peginterferon lambda.

Selling, general and administrative expenses

Selling, general and administrative expenses increased by $2.9 million to $20.8 million for the nine months ended September 30, 2022, from $17.9 million for the same period in 2021. The increase was primarily due to a $2.1 million increase in outside services, including consulting, advisory and accounting services, a $0.5 million increase in headcount related expenses, including stock-based compensation expense and a $0.3 million increase in other operating expenses, all to support company growth.

Interest expenses

Interest expense increased by $0.3 million compared to the same period in 2021. The increase was primarily due to an increase in outstanding principle balance related to the Innovatus loan.

Interest income

Interest income increased by $0.5 million compared to the same period in 2021. The increase was primarily due to an increase in the balance of money market funds and available-for-sale securities along with an increase in rates.

Other (expense) income, net

Other (expense) income, net changed by $47.5 million compared to the same period in 2021. The change was primarily due to $45.9 million net proceeds received from the sale of our rare pediatric disease priority review voucher to AbbVie Inc in 2021 and a $1.1 million loss on early extinguishment of the Oxford loan in 2022.

Provision for income taxes

Provision for income taxes decreased by $20,000 compared to the same period in 2021. The change was primarily due to the tax expense related to state taxes.

Liquidity and Capital Resources

Sources of Liquidity

As of September 30, 2022, we had $121.0 million of cash, cash equivalents and short-term debt securities, comprised of $26.3 million of cash and cash equivalents and $94.7 million of short-term debt securities available-for-sale, and an accumulated deficit of $412.1 million.

On June 1, 2022, we entered into a loan and security agreement with Innovatus, providing for up to $75.0 million funded in three tranches with a maturity date of August 31, 2027. We were funded $40.0 million in June 2022 at the closing, of which approximately $33.5 million was used to pay off the Oxford Loan. We recorded a loss of $1.1 million on early extinguishment of the debt which was recognized as a component of Other (expense) income, net in the condensed consolidated statement of operations and other comprehensive loss. As part of the agreement with Innovatus,we are required to maintain a cash balance of not less than 5% of the aggregate principal amount of funded and outstanding term loans at all times. Additionally, in connection with entering into the Innovatus Loan, we entered into a Stock Purchase Agreement with Innovatus for the sale of common stock with an aggregate value of $5.0 million. On June 1, 2022, we issued 749,053 shares of common stock to Innovatus at a per share purchase price of $6.6751.

On December 18, 2020, we filed a shelf registration statement on Form S-3 (File No. 333-251497) with the Securities and Exchange Commission (SEC), which was declared effective by the SEC on December 31, 2020 and permits the offer, issuance and sale by us up to a maximum aggregate offering price of $200.0 million of our common stock, preferred stock, debt securities and warrants. In connection with the filing of the registration statement, we entered into an Open Market Sale Agreement℠ with Jefferies LLC (Jefferies), pursuant to which we could sell up to a maximum of $50.0 million of our common stock in offerings that are seemed “at the market” offerings as defined in Rule 415 under the Securities Act (2020 ATM Facility). In March 2022, we completed the sale of all shares available under the 2020 ATM Facility, and the 2020 ATM Facility was terminated.

On March 25, 2022, we entered into a new Open Market Sale Agreement℠ with Jefferies, pursuant to which we can sell up to a maximum of $50.0 million of our common stock in offerings that are deemed “at the market” offerings as defined in Rule 415 under the Securities Act, under Eiger’s effective shelf registration statement (the 2022 ATM Facility). In April 2022, we completed offerings from the 2022 ATM facility for a total of 2,686,288 shares of our common stock resulting in net proceeds of $20.8 million, after deducting commissions costs. No additional offerings were completed since April 2022. As of September 30, 2022, there was approximately $28.7 million remaining under the 2022 ATM Facility for future issuance.

We believe that the 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. 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 selling, 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

Prior to 2021, we had not generated any product revenue. We launched our first commercial product, Zokinvy, in January 2021. 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 the development of our product candidates, sales and marketing costs for commercialization of Zokinvy and other product candidates, compensation and related expenses, hiring additional staff, including clinical, scientific, operational, manufacturing, 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, including pursuant to the 2022 ATM Facility, 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,	
	2022	2021
Net cash used in operating activities	$ (60,763)	$ (54,577)
Net cash (used in) provided by investing activities	(12,165)	74,865
Net cash provided by financing activities	77,015	103
Net increase in cash and cash equivalents	$ 4,087	$ 20,391

Cash flows from operating activities

Cash used in operating activities for the nine months ended September 30, 2022 was $60.8 million, which primarily consisted of a net loss of $71.6 million, stock-based compensation expense of $6.5 million, loss on extinguishment of long-term debt of $1.1 million, inventory write down of $1.0 million, non-cash interest expense of $0.9 million, amortization of debt securities discount of $0.7 million, amortization of operating lease right-of-use assets of $0.4 million and depreciation and amortization of $0.2 million. Additionally, cash used in operating activities reflected changes in net operating assets due to an increase of $4.1 million in accounts payable and accrued liabilities primarily due to the timing of payments and a decrease of $0.1 million in accounts receivable partially offset by an increase of $2.2 million in prepaid expenses and other current assets, an increase of $0.9 million in inventories, an increase of $0.6 million in other assets, and a decrease of $0.5 million in operating lease liabilities.

Cash used in operating activities for the nine months ended September 30, 2021 was $54.6 million, which primarily consisted of a net loss of $12.1 million, a $46.5 million gain from sale of priority review voucher and $0.3 million income related to asset purchase agreement which were partially offset by stock-based compensation expense of $5.9 million, non-cash interest expense of $0.6 million, amortization of debt securities premiums and discounts of $0.7 million, amortization of operating lease right-of-use assets of $0.4 million, depreciation and amortization of $0.2 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 $3.6 million due to an increase of $2.5 million in inventories, an increase of $2.8 million in accounts receivable, a decrease of $0.4 million in operating lease liabilities, and an increase of $0.8 million in other assets, partially offset by an overall increase of $2.4 million in accounts payable and accrued liabilities primarily due to the timing of payments, and by a decrease of $0.5 million in prepaid expenses and other current assets.

Cash flows from investing activities

Cash used in investing activities for the nine months ended September 30, 2022 was $12.2 million, primarily consisting of $55.5 million of purchases of debt securities, which were partially offset by $43.5 million of proceeds from maturities of debt securities.

Cash provided by investing activities was $74.9 million for the nine months ended September 30, 2021, primarily consisting of $99.6 million of proceeds from maturities of debt securities, $46.5 million of net proceeds received from the sale of our priority review voucher, and $0.2 million of proceeds pursuant to our asset purchase agreement with Theragene, which were partially offset by $71.4 million of purchases of debt securities.

Cash flows from financing activities

Cash provided by financing activities for the nine months ended September 30, 2022 was $77.0 million, which primarily consisted of $66.4 million of proceeds net of commissions from the issuance of common stock under the 2020 and 2022

ATM Facilities, $39.8 million of proceeds from debt, $5.0 million of proceeds from issuance of common stock to lender, $0.3 million proceeds from issuance of common stock upon stock option exercises and ESPP purchases, which were partially offset by the $33.3 million repayment of debt, $1.1 million payment of debt issuance costs and $0.2 million of common stock offering costs.

Cash provided by financing activities for the nine months ended September 30, 2021 primarily consisted of $0.5 million of proceeds from the issuance of common stock upon stock option exercises and ESPP purchases, partially offset by $0.2 million payments for debt issuance costs and $0.2 million payments for deferred offering costs.

Contractual Obligations and Other Commitments

Our contractual obligations as of September 30, 2022 have not materially changed from what we presented in our Annual Report on Form 10-K for the year ended December 31, 2021 filed with the SEC on March 10, 2022, other than our outstanding debt balance as described above.

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, 2022, the end of the period covered by this report.

Changes in 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 nine months ended September 30, 2022 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 10-Q, 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 10-Q and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risk Factor Summary

Below is a summary of material factors that make an investment in our common stock speculative or risky. Importantly, this summary does not address all of the risks and uncertainties that we face. Additional discussion of the risks and uncertainties summarized in this risk factor summary, as well as other risks and uncertainties that we face, can be found under “Risk Factors” in Part II, Item 1A of this Quarterly Report on Form 10-Q. The below summary is qualified in its entirety by that more complete discussion of such risks and uncertainties. You should carefully consider the risks and uncertainties described under “Risk Factors” in Part II, Item 1A of this Quarterly Report on Form 10-Q as part of your evaluation of an investment in our common stock.

- Ability to develop peginterferon lambda for COVID-19 or file for a temporary use authorization with a foreign regulatory agency.
- We are a commercial-stage biopharmaceutical company with additional product candidates in clinical development and a limited operating history. We have incurred net losses in each year since our inception. We have one FDA and EMA-approved product for commercial sale, Zokinvy (lonafarnib), and prior to 2021, have never generated any product revenue and may never be profitable.
- We are dependent on the success of our product candidates, which are in various stages of clinical development. We cannot give any assurance that we will generate data for any of our product candidates sufficient to receive regulatory approval and without regulatory approval we will not be able to market our product candidates.
- Prior to the approval of our new drug application (NDA) for Zokinvy® (lonafarnib) to reduce the risk of mortality in progeria, and for treatment of processing-deficient progeroid laminopathies with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, we had not submitted an application for approval or obtained U.S. Food and Drug Administration (FDA) approval for any product. We may not be able to obtain FDA approval of any future NDA or Biologics License Application (BLA) for our product candidates, which would prohibit commercialization.
- Our business strategy is based upon obtaining and maintaining Orphan Drug designation for our product candidates. If we are unable to obtain or maintain Orphan Drug designation or regulatory approval, our business would be substantially harmed.
- 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.
- We rely on clinical studies of our product candidates in order to obtain regulatory approval. 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.
- If clinical studies of our product candidates fail to demonstrate safety and efficacy to the satisfaction of the FDA or similar regulatory authorities outside the United States or do not otherwise produce positive results, we may

incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

- We rely on third parties to conduct our clinical studies, manufacture our product candidates and perform other services. Our ability to obtain regulatory approval or commercialize our product candidates and our business could be impaired if these collaborations are unsuccessful.
- If the market opportunities for our product candidates are smaller than we believe they are, we may not meet our revenue expectations. 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 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.
- We currently have limited marketing and sales capabilities for the commercialization of our product candidates.
- 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. Sales of our products depend substantially on the extent to which the costs of our product candidates will be paid for or reimbursed by healthcare management organizations or government authorities or third-party payors.
- We are currently conducting and will continue to conduct clinical trials for our product candidates outside the United States, which could expose us to risks that could have a material adverse effect on our business, including risks in connection with the actions taken by the Russian Federation in Ukraine and surrounding areas.
- We intend to rely on a combination of exclusivity from Orphan Drug designation and our patent rights for our product candidates. If we are unable to maintain exclusivity from the combination of these approaches, then our ability to compete effectively in our markets may be harmed.
- The annual reassessment by the EMA of the risk-benefit balance for Zokinvy including information on the safe and effective use may not be positive, which could lead to a variation, suspension, revocation of our marketing authorization or requirement to fulfil additional specific obligations.
- If we are unable to maintain effective proprietary rights for our product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize product candidates similar or identical to ours.
- We may be unsuccessful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses. 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 may not be successful in any efforts to identify, license, discover, develop or commercialize additional product candidates.
- Healthcare legislative reform measures may have a material adverse effect on our business and 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.
- We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs.
- We are dependent upon information technology systems and any failure or security breach of such systems could result in a material disruption in the development of our product candidates or other business operations as well as result in statutory or contractual obligations or otherwise expose us to liability.
- The current COVID-19 pandemic has and may continue to adversely affect our financial condition and our business as well as those of third parties on which we rely on significant manufacturing, clinical or other business operations.

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 commercial-stage biopharmaceutical company with a limited operating history. We have incurred net losses in each year since our inception. For the three months ended September 30, 2022 and 2021, we reported a net loss of $27.1 million and $22.2 million, respectively. For the nine months ended September 30, 2022 and 2021, we reported a net loss of $71.6 million and $12.1 million, respectively. As of September 30, 2022, we had an accumulated deficit of $412.1 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 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 selling, 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 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-based regimens for use in an HDV indication. We may need significant additional resources in order to aggressively move lonafarnib-based regimens forward successfully based on the discussions with the FDA. It may be several years, if ever, before we complete pivotal clinical studies and have additional product candidates 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 one or more additional product candidates, 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 Zokinvy available to progeria (HGPS and processing-deficient progeroid laminopathies) patients under an expanded access program that may not result in payment to us. Future clinical trials of new therapies for progeria conducted by third parties may also result in patients converting from commercially reimbursed Zokinvy to product provided through clinical trials and result in lower revenues received by 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;
- commercialize and provide expanded access to Zokinvy for the treatment of progeria
- identify and develop potential commercial opportunities, such as lonafarnib-based regimens, peginterferon lambda for HDV, and avexitide for HI and PBH;

- 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.

Prior to 2021, we never generated any product revenue and may never be profitable.

We have one product approved for commercialization in the U.S. and EU for two ultra-rare diseases. Zokinvy to reduce the risk of mortality in HGPS, and for treatment of processing-deficient progeroid laminopathies with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, was approved by the FDA in November 2020 and launched commercially in the U.S. in January 2021, under the exceptional circumstances procedure and our MAA for Zokinvy was approved by the EC, based on a recommendation by the EMA, in July 2022. The MA is subject to the EMA's continued review on an annual basis of new efficacy and safety information which may become available. Our ability to generate substantial revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to obtain the regulatory and marketing approvals necessary to commercialize Zokinvy in foreign jurisdictions and to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize, more of our product candidates in the U.S. or foreign jurisdictions. We do not anticipate generating significant product revenue for the foreseeable future. Our ability to generate future product revenue depends heavily on our success in many areas, including but not limited to:

- completing research and development of our product candidates;
- obtaining additional and maintaining current 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 we obtain additional product approvals 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 sufficient 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, including pursuant to the 2022 ATM Facility, 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 Innovatus Life Sciences Lending Fund I, LP (Innovatus) in June 2022 (the Innovatus Loan). The Innovatus Loan was a $75.0 million debt financing arrangement with Innovatus wherein we borrowed the first tranche of $40.0 million upon closing of the debt financing in June 2022. The Innovatus Loan is secured by perfected first priority liens on our assets. The Innovatus 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 worsening global economic conditions and continuing 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 Innovatus 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 Innovatus Loan provides for up to $75.0 million in term loans due on August 31, 2027, of which $40.3 million in principal is outstanding as of September 30, 2022. All of our current and future assets, secure our borrowings under the Innovatus Loan. The Innovatus 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 Innovatus 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 Innovatus 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 Innovatus 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.

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 further develop, obtain regulatory approvals for, and commercialize one or more of these product candidates. Our NDA for Zokinvy to reduce the risk of mortality in HGPS, and for treatment of processing-deficient progeroid laminopathies (PL) with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, was approved in November 2020. Prior to the U.S. Zokinvy commercial launch in 2021, we had not generated revenue from sales of any drugs and may never be able to develop or commercialize additional product candidates. In addition, we have a commitment to provide access to Zokinvy for patients with HGPS and PL with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, for no or minimal cost to those patients.

With respect to potential commercial products, we currently have three product candidates that are in Phase 3 clinical development, lonafarnib-based regimens for HDV, peginterferon lambda for HDV, and avexitide for HI development program. Avexitide for PBH has completed Phase 2 clinical trials. It may be years before our studies are completed, and new studies initiated, 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. Likewise, there can be no assurance that the data obtained from foreign clinical trial sites in studies not conducted under an investigational new drug application, or IND, will be accepted in support of an application for regulatory approval or authorization for use in the U.S. Similarly, data obtained from foreign clinical trial sites may not be accepted by other foreign regulatory authorities in support of an application for regulatory approval or authorization for use in these jurisdictions.

- 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 current 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 any future NDA or BLA 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-based regimens, peginterferon 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 approval of our NDA for Zokinvy to reduce the risk of mortality in HGPS, and for treatment of PL with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, we had not submitted an application for approval or obtained FDA approval for any product.

Approval of an NDA or a BLA 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. In previous studies, ECG abnormalities were observed in our lonafarnib program. 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 or maintain 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 obtained U.S. regulatory approval for one product, Zokinvy, and it is possible that none of our current product candidates or any future product candidates we may seek to develop will ever obtain regulatory approval.

Future 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 an NDA,BLA, 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 additional 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 avexitide for the treatment of congenital hyperinsulinism, NDA approval for this program may not meet the eligibility criteria for a priority review voucher.

Our avexitide compound has received Rare Pediatric Disease (RPD) designation from the FDA for the treatment of Congenital Hyperinsulinism (HI). The FDA defines a "rare pediatric disease" as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect patients under the age of 18 years, that is, a disease or

condition that affects fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States when there is no reasonable expectation that the cost of developing and making available the drug in the United States will be recovered from sales in the United States for that drug or biological product. 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. While we obtained and sold the priority review voucher issued upon approval of Zokinvy to reduce the risk of mortality in HGPS, and for treatment of PL with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, there can be no assurance that we will be successful in obtaining approval for avexitide for the treatment of HI, or that a priority review voucher will be issued at the time of any such approval.

Congress has only authorized the Rare Pediatric Disease Priority Review Voucher Program until September 30, 2024. However, if a drug candidate receives RPD designation before September 30, 2024, it is eligible to receive a voucher if it is approved before September 30, 2026. 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.

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 Designations, this may not lead to a faster development, regulatory review or approval process, and it does not increase the likelihood of receiving marketing approval in the United States.

We have received Breakthrough Therapy Designation for lonafarnib and peginterferon lambda for the treatment of HDV, and for avexitide for the treatment of HI and PBH. 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.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. 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. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product no longer meets the conditions for qualification. Thus, even though we intend to seek Breakthrough Therapy Designation for and some or all of our future product candidates for the treatment of various cancers, there can be no assurance that we will receive any such Breakthrough Therapy Designation.

We may submit an NDA or BLA for our product candidates under the Accelerated Approval Pathway. If we are unable to obtain approval of our drug candidates through the Accelerated Approval Program in the United States, we may be required to conduct additional nonclinical and clinical studies and trials beyond those that we currently contemplate, which could increase the expense of obtaining, reduce the likelihood of obtaining and/or delay the timing of obtaining, necessary marketing approval. Even if we receive approval from the FDA through the Accelerated Approval Program, if any required confirmatory post-marketing trial does not verify clinical benefit, or if we do not comply with rigorous post-marketing requirements, the FDA may seek to withdraw the approval.

We may seek approval under the Accelerated Approval pathway for our lonafarnib and peginterferon lambda products for the treatment of HDV. For any approval to market a product, we must provide the FDA and foreign regulatory agencies with clinical data that adequately demonstrate the safety and efficacy of the product for the indication applied for in the NDA, BLA, or other respective regulatory filings. The Accelerated Approval Program is one of several approaches used by the FDA to make prescription drugs and biologics more rapidly available for the treatment of serious or life-threatening diseases. Section 506(c) of the Federal Food, Drug and Cosmetic Act (FDCA) provides that the FDA may grant accelerated approval to "a product for a serious or life-threatening condition upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments." Approval through the Accelerated Approval Program is typically subject, however, to the requirement that the applicant conduct additional post-marketing clinical trials to verify and describe the product's clinical benefit. Typically, clinical benefit is verified when post-marketing clinical trials show that the product provides a clinically meaningful positive therapeutic effect, that is, an effect on how a patient feels, functions, or survives. If such confirmatory post-marketing trial fails to confirm the product's clinical profile or risks and benefits, the FDA may withdraw its approval of the product.

The FDA has broad discretion with regard to approval through the Accelerated Approval Program, and even if we believe that the Accelerated Approval Program is appropriate for our product candidates, we cannot assure you that the FDA will ultimately agree. Furthermore, even if we do obtain approval through the Accelerated Approval Program, we may not experience a faster development process, review or approval compared to conventional FDA procedures.

Our lonafarnib-based regimens and peginterferon lambda products for the treatment of HDV are in Phase 3 studies with endpoints intended to support accelerated approval. The primary endpoint for the D-LIVR study, the Phase 3 study of lonafarnib-based regimens, is a composite of a > 2 log reduction in HDV RNA and ALT normalization and is intended to support accelerated approval. The primary endpoint for the LIMT-2 study, the Phase 3 study of peginterferon lambda, is a durable virologic response (DVR), defined as HDV RNA below the limit of quantitation at 24 weeks post-treatment, and is intended to support accelerated approval for finite therapy. The study endpoints were previously achieved in Phase 2 studies and are consistent with FDA guidance on the development of treatments for HDV. While these proposed endpoints are designed to be consistent with FDA guidance, there is no assurance that approval will be granted on a timely basis, or at all. FDA may disagree that the design of, or results from, our studies support accelerated approval. Additionally, the FDA could require us to conduct further studies or trials prior to granting approval of any type, including by determining that approval through the Accelerated Approval Program is not appropriate and that our clinical trials may not be used to support approval through the conventional pathway. We might not be able to fulfill the FDA's requirements in a timely manner, which would cause delays, or approval might not be granted because our submission is deemed incomplete by the FDA. There also can be no assurance that after subsequent FDA feedback we will continue to pursue approval through the Accelerated Approval Program. A failure to obtain approval through the Accelerated Approval Program could result in a longer time period to obtain approval of our product candidates, could increase the cost of their development, could delay our ability to commercialize our products and could significantly harm our financial position and competitive position in the marketplace.

Even if we receive approval for one or more of our product candidates through the Accelerated Approval Program, we will be subject to rigorous post-marketing requirements, possibly including the completion of one or more confirmatory post-marketing trials as the FDA may require, to verify the clinical benefit of the product, and submission to the FDA of all promotional materials prior to their dissemination. The FDA could seek to withdraw the approval for multiple reasons, including if we fail to conduct any required confirmatory post-marketing trial with due diligence, our confirmatory post-marketing trial does not confirm the predicted clinical benefit, other evidence shows that the product is not safe or effective under the conditions of use, or we disseminate promotional materials that are found by the FDA to be false and misleading.

Moreover, Congress is considering potential changes to the Accelerated Approval Program that could impact our ability to obtain accelerated approval, or increase the burdens associated with post marketing requirements in the event we do obtain accelerated approval. It is not possible to predict which changes Congress will include if it decides to enact changes to the Accelerated Approval Program, or if Congress will make any changes to the Accelerated Approval Program at all.

Any delay in obtaining, or inability to obtain, approval through the Accelerated Approval Program, or any issues in maintaining approval granted under the Accelerated Approval Program, would delay or prevent commercialization of our products, and would materially adversely affect our business, financial condition, results of operations, cash flows and prospects

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 in our clinical trials as a result of the evolving COVID-19 global pandemic and competition for HDV patients at our European clinical trial sites due to the conditional approval of bulevirtide in Europe and potential competition at our U.S. sites if bulevirtide receives FDA approval. For example, early in the pandemic, certain clinical study sites that were scheduled to open were delayed in activating and other sites suspended randomization of subjects for a period of time. Future pandemic restrictions could result in delays of our clinical trials, including D-LIVR and LIMT-2. 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;
- feasibility of continuous trial execution in countries impacted by war, geopolitical conflict and other humanitarian crises;
- 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 good clinical practice (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 or maintain Orphan Drug designation exclusivity and to successfully commercialize our product candidates and may harm our business and results of operations.

We may not be able to receive an Emergency Use Authorization (EUA) from FDA in the U.S., or similar temporary use authorization from foreign regulatory agencies. If we do receive an EUA or similar temporary use authorization, absent a full marketing authorization for that indication, our ability to sell our products would be revoked when the COVID-19 public health emergency terminates.

On October 5, 2022, we announced that, following feedback from FDA, we will not submit an EUA application for peginterferon lambda for the treatment of patients with mild-to moderate COVID-19. If we decide to submit a future application for an EUA we cannot predict whether FDA will grant an EUA . If we do not receive an EUA from FDA, we will not be able to commercialize future products and may be required to conduct additional clinical trials for an EUA. Obtaining such an authorization is dependent upon a number of factors, which are not under our control. Even if an EUA is received, we also cannot predict how long, if ever, an EUA would remain in place.

We do intend to pursue similar temporary use authorizations in non-U.S. jurisdictions.

Various regulatory pathways are available in jurisdictions outside the United States to make drugs available for emergency use. For example, regulatory authorities in certain European Union Member States may temporarily authorize the distribution of an unauthorized drug in response to the suspected or confirmed spread of pathogenic agents such as the virus which is causing COVID-19. Obtaining such a temporary authorization is dependent upon a number of factors, which are not under our control. If such authorizations would be granted, they would only apply for a limited period of time. We

might thus no longer be authorized to distribute our drug under these authorizations if that time limit expired or the pandemic terminates.

The regulatory authorities in the European Union or in other jurisdictions outside the US may grant a conditional marketing authorization for medicinal products intended for the treatment of seriously debilitating or life-threatening diseases prior to the submission of comprehensive clinical data if that treatment is of major therapeutic advantage to the patients concerned or no other authorized treatment is available. In emergency situations, such a conditional marketing authorization may also be granted for these medicinal products where comprehensive pre-clinical or pharmaceutical data have not been supplied. These conditional marketing authorizations are subject to specific conditions (*e.g.*, completing on-going studies or conducting new studies) which must be fulfilled within a timeline specified in the marketing authorization. These marketing authorizations are valid for a short period of time (*e.g.*, one year) which can usually be renewed.
If we would apply for such a conditional marketing authorization the regulatory authority concerned may reject our application because it considers that the benefit-risk balance of our product is not favorable or it judges it unlikely that we would be able to provide comprehensive data. If we would obtain such a conditional marketing authorization we may not be able to complete (timely) the studies which the regulatory authority imposed as a condition for the marketing authorization or the data collected in the course of these studies may indicate that our drug does not have a favorable benefit-risk profile. As a result we may not be able to continue distributing our drug because the conditional marketing authorization has been revoked or not renewed, or the regulatory authority refused finally to grant a regular marketing authorization.

Regulatory authorities in the EU or other jurisdictions outside the EU may make a drug available for compassionate reasons to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening, and who cannot be treated satisfactorily with an authorized medicinal product provided that the drug is subject of an application for marketing authorization or is undergoing clinical trials. If we would request a regulatory authority to make our drug available to patient under these conditions the regulatory authority may reject our request if, for example, it considers that the patients concerned can be treated satisfactorily with other products that are already authorized.

Programs which make drugs available under the conditions mentioned above are usually authorized for a limited period of time (*e.g.*, one year) . Regulatory authorities may not renew expiring authorizations for these programs if we terminate prematurely a clinical trial with our drug or decide not to submit or to withdraw an application for marketing authorization in the jurisdiction concerned. That may, for example, happen because pharmacovigilance data or other data collected during our clinical trials indicate that our drug does not have the appropriate benefit-risk balance.

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. Use of Zokinvy to reduce the risk of mortality in HGPS, and for treatment of processing-deficient progeroid laminopathies with either heterozygous LMNA mutation with progerin-like protein accumulation, or homozygous or compound heterozygous ZMPSTE24 mutations, 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 peginterferon 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 peginterferon 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-based regimens for HDV. Our avexitide product candidate has been studied in 39 HI patients and 54 PBH 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 current 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.

We are subject to ongoing regulatory requirements related to the U.S. and European approvals of Zokinvy, and if we obtain additional regulatory approvals for a product candidate, we will be subject to additional 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, BLA, 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 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, ongoing progeria clinical studies and expanded access program, and commercial Zokinvy supply 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, EMA or others, our 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, and in the past we have experienced quality control issues with product manufactured by our contract manufacturers. 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 not be able to develop additional commercially viable products.

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, although we believe that our lonafarnib-based regimens and peginterferon lambda 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, we expect that the sales of Zokinvy to patients with progeria will have limited profits given the ultra-rare nature of these diseases.

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, Johnson & Johnson, Replicor, Arrowhead Pharmaceuticals, Novartis International, Zealand Pharmaceuticals, Xeris Pharmaceuticals, Rezolute, Hanmi Pharmaceutical, and Crinetics Pharmaceuticals 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.

Although we commercially launched Zokinvy following its FDA approval, we 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 limited recent experience selling and marketing our product candidates and we currently have a small sales and marketing organization. To successfully commercialize Zokinvy and additional products that may result from our development programs, we will need to invest in and expand 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 recent, limited 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 Zokinvy available for patients with progeria, 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 timing of our receipt of any marketing and commercialization licensures;
- the terms of any licensures and the countries in which licensures are obtained;
- 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 or any new methods of administration;
- the marketing, sales and distribution support for the product;
- the publicity concerning our products or competing products and treatments;
- the success of our physician education programs;
- availability of alternative effective treatments for the disease indications our product candidates are intended to treat and the relative risks, benefits and costs of those 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 products 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, Zokinvy for patients with Progeria and processing-deficient 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 (HHS), as CMS decides whether and to what extent a new drug will be covered and reimbursed under Medicare. Private payors often follow the coverage reimbursement policies established by CMS to a substantial degree. It is difficult to predict what CMS and other payors will decide with respect to coverage for products such as ours and what reimbursement our products may receive.

The process for determining whether a third-party payor will provide coverage for a drug product typically is separate from the process for setting the price of a drug product or for establishing the reimbursement rate that the payor will pay for the drug product once coverage is approved. Third-party payors may limit coverage to specific drug products on an approved list, also known as a formulary, which might not include all of the FDA-approved drugs for a particular indication. A decision by a third-party payor not to cover Zokinvy or any of our product candidates once approved could reduce utilization of such products and have a material adverse effect on our sales, results of operations and financial condition. Moreover, a third-party payor’s decision to provide coverage for a drug product does not imply that adequate reimbursement will be available. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. Additionally, coverage and reimbursement for drug products can differ significantly from payor to payor. One third-party payor’s decision to cover a particular medical product or service does not ensure that other payors will also provide coverage for the medical product or service or will provide coverage at an adequate reimbursement rate. As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our products to each payor separately and will be a time-consuming process.

Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost-effectiveness of drug products and medical services, in addition to questioning safety and efficacy. If third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after FDA approval or, if they do, the level of payment may not be sufficient to allow us to sell our products at a profit. Additionally, coverage policies and third-party reimbursement rates may change at any time. Therefore, even if favorable coverage and reimbursement status is attained for one or more of our products, less favorable coverage policies and reimbursement rates may be implemented in the future. If we are unable to obtain and maintain sufficient third-party coverage and adequate reimbursement, the commercial success of our products may be greatly hindered and our financial condition and results of operations may be materially and adversely affected.

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, inadequate coverage or payment for our products.

We expect to experience pricing pressures in connection with products due to increased and continued efforts to limit or reduce healthcare spending. 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. 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.

Congress is considering updates to the orphan drug provisions of the FDCA in response to a recent decision by the U.S. Court of Appeals for the 11th Circuit. Any changes to the orphan drug provisions could change our opportunities for, or likelihood of success in obtaining, orphan drug exclusivity and would materially adversely affect our business, financial condition, results of operations, cash flows and prospects.

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 or maintain 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.

The annual reassessment by the EMA of the risk-benefit balance, including information on the safe and effective use, for Zokinvy may not be positive, leading to a variation, suspension, revocation of our MA or fulfilment of additional specific obligations.

On July 20, 2022, we announced that the EC granted an MA under the exceptional circumstances procedure for Zokinvy to treat patients aged 12 months and older with HGPS and PL. The EC authorization follows the positive opinion granted by the CHMP in May 2022 which found that the risk-benefit balance for Zokinvy is favorable to recommend the granting of a MA although the rarity of the disease means that it was not possible to obtain complete information on Zokinvy during the assessment process. As a result, the MA was issued under the exceptional circumstances procedure and subject to the EMA’s continued review on an annual basis of new efficacy and safety information which may become available.

Under Article 14(8) Regulation (EC) No 726/2004, products for which the applicant can demonstrate that comprehensive data (in line with the requirements laid down in Annex I of Directive 2001/83/EC, as amended) cannot be provided (due to

specific reasons foreseen in the legislation) might be eligible for marketing authorization under exceptional circumstances. This type of authorization is reviewed annually to reassess the risk-benefit balance. The fulfillment of any specific procedures/obligations imposed as part of the marketing authorization under exceptional circumstances is aimed at the provision of information on the safe and effective use of the product.

It is possible that the annual reassessment by the EMA of the risk-benefit balance including information on the safe and effective use for Zokinvy may not be positive. This could lead to the variation, suspension, revocation of our MA for Zokinvy in the EU, or lead to additional specific commitments or conditions being fulfilled.

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-based regimens, peginterferon 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 license agreement for lonafarnib. We may not be able to achieve the required diligence milestones in a timely manner, which may result in the license agreement being terminated, 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 marketed under NDAs 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 marketed under an NDA. Generally, in place of clinical studies intended to demonstrate safety or effectiveness, an ANDA applicant usually needs only to submit data demonstrating that its product has the same active ingredient(s), strength, dosage form, route of administration and that it is bioequivalent to the branded 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. The FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity (NCE). During the exclusivity period, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company for another version of such product candidate where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an approved NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing product candidate. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for product candidates containing the original active agent for other conditions of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the nonclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Manufacturers may seek to launch these generic drugs following the expiration of the marketing exclusivity period, even if we still have patent protection for our drug. Competition that our drug candidates may face from generic versions of our drug candidates could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those drug candidates. Our future revenues, profitability and cash flows could also be materially and adversely affected and our ability to obtain a return on the investments we have made in those drug candidates may be substantially limited if our drug candidates, if and when approved, are not afforded the appropriate periods of non-patent exclusivity.

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.

Our biological product candidates for which we intend to seek licensure may face competition from biosimilars.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated licensure pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the licensure of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our product candidates.

There is a risk that any product candidates we may develop that are licensed as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider any product candidates we may develop to be reference products for competing products, potentially creating the opportunity for competition sooner than anticipated. In addition, the approval of a biologic product biosimilar to one of our products could have a material adverse impact on our business as it may be significantly less costly to bring to market and may be priced significantly lower than our products. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation, including litigation challenging the constitutionality of the ACA. For example, in December 2018, a federal district court ruled that the ACA, without the "individual mandate" penalty (which was repealed by Congress as part of the Tax Cuts and Jobs Act), is unconstitutional in its entirety. In December 2019, the U.S. Court of Appeals for the 5th Circuit upheld the district court ruling that the individual mandate provisions are unconstitutional and remanded the case back to the district court for further analysis of whether such provisions could be severed from the remainder of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the case without specifically ruling on the constitutionality of the ACA. There may, however, be other efforts to challenge, repeal, or replace the ACA in the future. We continue to evaluate the effect that the ACA and its possible repeal and replacement has (or may have) on our business and exclusivity under the BPCIA. It is uncertain the extent to which any such changes may impact our business or financial condition.

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, 2022, we had 54 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 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 significant penalties if we obtain, use, or disclose individually identifiable health information in a manner that is not authorized or permitted by HIPAA. On December 10, 2020, the Office of Civil Rights within the Department of Health and Human Services issued proposed revisions to the HIPAA Privacy Rule aimed at reducing regulatory burdens that may exist in discouraging coordination of care and patient access to their health information, among other changes. While a final rule has not yet been issued, if adopted, these proposed changes may require us to update our policies and procedures to comply with the new requirements.

In particular, several state laws have recently been passed or amended to significantly expand privacy rights and obligations of businesses that process personal information, including identifiable sensitive health information. For example, 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. The California Privacy Rights Act of 2020 (CPRA), which expands the CCPA, was passed in the election on November 3, 2020. The CPRA will, among other things, give consumers the ability to limit use of information deemed to be sensitive, increase the maximum penalties for violations concerning consumers under age 16, and establish the California Privacy Protection Agency to implement and enforce the new law and impose administrative fines. Following the CPRA, Virginia and Colorado have enacted similar, but not completely consistent, comprehensive privacy legislation that will also go into effect in January and July 2023, respectively. Many other states are considering similar legislation.

Aspects of the these new state privacy laws, and their interpretation and enforcement, remain uncertain. The potential effects of these new and evolving state privacy laws are far-reaching and may require us to modify our data processing practices and policies and to incur substantial costs and expenses in an effort to comply. Although the California, Virginia and Colorado laws include 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, and those health-data related exceptions may evolve through amendment or regulatory interpretation. The state privacy law developments, moreover, have prompted a number of additional 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.

Foreign data protection laws, including, without limitation, the EU GDPR that took effect in May 2018, and EU Member State data protection legislation, may also apply to health-related and other personal information obtained from individuals. The EU GDPR has been transposed into the national laws of the United Kingdom by virtue of section 3 of the European Union (Withdrawal) Act 2018 as the UK GDPR (together, the EU GDPR and the UK GDPR, the GDPR). The EU GDPR has direct effect in all EU Member States and has extraterritorial effect where organizations outside of the EU process personal information of individuals in the EU in relation to the offering of goods or services to those individuals (targeting test) or the monitoring of their behavior (monitoring test). The UK GDPR has a similar extraterritorial test for organizations outside of the UK processing personal information of individuals in the UK in relation to the offering of goods or services to those individuals or the monitoring of their behavior. As such, the GDPR applies to us to the extent we are established in an EU Member State or the UK or we meet the requirements of either the targeting test or the monitoring test. These laws impose strict obligations on businesses, including to: (i) implement administrative, physical, technical, and organizational safeguards to protect personal information; (ii) establish an appropriate and valid legal basis for processing personal information; (iii) comply with accountability transparency requirements regarding the processing of personal information, which require controllers to demonstrate and record compliance with the GDPR and to provide more detailed information to data subjects regarding processing; (iv) comply with data protection rights of data subjects including a right of access to and rectification of personal information, a right to obtain restriction of processing or to object to processing of personal information, a right to ask for a copy of personal information to be provided to a third party in a useable format and erasing personal information in certain circumstances; (v) report certain personal data breaches to the relevant supervisory authority without undue delay (and no later than 72 hours, where feasible); (vi) obtain explicit consent for collection of sensitive personal information such as health data; and (vii) consider data protection as any new products or services are developed and to limit the amount of personal information processed.

The EU GDPR restricts the transfer of personal information from the European Economic Area (EEA) to the United States and other countries that the European Commission does not recognize as having “adequate” data protection laws unless the parties to the transfer have implemented an appropriate data transfer mechanism in accordance with the EU GDPR. The UK GDPR has similar restrictions on transfers of personal information from the United Kingdom to countries that the UK does not recognize as having “adequate” data protection laws in the United Kingdom (as discussed below) and Switzerland impose similar restrictions. One of the primary mechanisms allowing United States companies to import personal information from Europe had 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, in July 2020, the Court of Justice of the EU (CJEU) invalidated the EU-U.S. Privacy Shield, and subsequent regulatory guidance required additional compliance efforts to analyze international data flows and take steps to ensure adequate protections for personal data transferred to the United States and other certain jurisdictions, including by implementing supplementary measures that provide privacy protections in addition to those provided under the Standard Contractual Clauses (SCCs). Moreover, new versions of the European Commission’s Standard Contractual Clauses, now the primary mechanism for the lawful transfer of personal information transfers from Europe and/or the United Kingdom to the United States or other countries, have been released requiring additional compliance and implementation efforts. The United Kingdom is also expected to publish its own set of SCCs in early 2022 for transfers of personal data outside of the United Kingdom. Similarly, the Swiss Federal Data Protection and Information Commissioner announced that the Swiss-U.S. Privacy Shield Framework is inadequate for personal information transfers from Switzerland to the United States in light of the CJEU’s July 2020 decision, and also raised questions about the viability of the older version of the Standard Contractual Clauses. As such, any transfers by us or our vendors of personal information from Europe may not comply with European data protection law, may increase our exposure to the EU GDPR’s heightened sanctions for violations of its cross-border data transfer restrictions and may reduce demand from companies subject to European data protection laws.

Moreover, where we rely on SCCs, we must now evaluate and implement supplementary measures that provide privacy protections additional to those provided under SCCs. This evaluation will, in particular, include an assessment as to whether the types of personal data transferred pursuant to SCCs may be subject to government surveillance in the data importer’s country and an assessment as to whether the data importer can meet its contractual obligations under the SCCs. This may have implications for our cross-border data flows and may result in compliance costs. Inability to import personal information from the EEA, 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 continue to enact 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. For example, Brazil enacted the General Data Protection Law (Lei Geral de Proteção

de Dados Pessoais or LGPD) (Law No. 13,709/2018), which broadly regulates the processing of personal information and imposes compliance obligations and penalties comparable to those of the EU GDPR.

Under the EU GDPR, regulators may impose substantial fines and penalties for non-compliance. Companies that violate the EU GDPR can face fines of up to the greater of 20 million Euros or 4% of their consolidated worldwide annual turnover (revenue) and restrictions or prohibitions on data processing. The EU also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the EU GDPR. The EU GDPR has increased our responsibility and liability in relation to personal information that we process, requiring us to put in place additional mechanisms to ensure compliance with the EU GDPR and other EU and international data protection rules. There may also be a risk that the measures will not be implemented correctly or that individuals within the business will not be fully compliant with the required procedures. Similarly, the UK GDPR introduces fines of up to the greater of £17.5 million or up to 4% of their consolidated worldwide annual turnover (revenue).

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, and our third-party vendors', 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, we and our vendors frequently defend against and respond to data security attacks and incidents, and vendors' servers are potentially vulnerable to physical or electronic break-ins, computer viruses, software vulnerabilities, ransomware attacks 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 financial motivation prompted by the enormous growth in ransomware over the past several years) 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 adequately prevent, detect or respond to data security breaches or privacy violations, or implement satisfactory remedial measures in the wake of a data security incident, 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, or our clinical trials may be adversely impacted from data loss resulting in delayed regulatory approvals or other operational impacts. 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 or other remote working activity that access and process confidential information remotely 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.

For example, in March 2021, we learned we were the victim of a business email compromise during which an unauthorized party gained access to the email account of an employee in our finance department. The incident resulted in a net loss of approximately $0.3 million. We immediately notified law enforcement and relevant banks involved in the wire, which we are working to recover. At this time, we do not know if we will be able to recover this loss, which we understand was transferred to another country. Based on our investigation, the incident was financially motivated and impacted a single email account. In response to the incident, we conducted a review of our corporate information technology and email policies and have implemented additional security measures.

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.

The financial exposure from the events referenced above could either not be insured against or not be fully covered through any insurance that we maintain. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages as a result of the events referenced above.

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 reform the delivery and payment for healthcare items and services. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (the 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. This law was designed to expand access to health insurance coverage for uninsured and underinsured individuals while at the same time containing overall healthcare costs. The framework of the ACA and other healthcare reforms continues to evolve as a result of executive, legislative, regulatory, and administrative developments. 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. In addition, the 2020 federal spending package permanently eliminated, 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 eliminated the health insurer tax. . There have also been numerous historical challenges and amendments to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Prior to the Supreme Court's decision, on January 28, 2021, President Biden issued an executive order instructing certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is unclear how the healthcare reform measures of the Biden administration or other efforts, if any, to challenge, repeal, amend or replace the ACA will impact the ACA and our business.

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 Congressional and federal agency inquiries regarding pricing and related practices, as well as proposed and enacted federal and state legislation and regulation 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 American Rescue Plan Act of 2021 included among its provisions a sunset of the ACA's cap on pharmaceutical manufacturers' rebate liability under the Medicaid Drug Rebate Program. Under the ACA, manufacturers' rebate liability was capped at 100% of the average manufacturer price for a covered outpatient drug. Effective January 1, 2024, manufacturers' Medicaid Drug Rebate Program rebate liability will no longer be capped, potentially resulting in a manufacturer paying more in Medicaid Drug Rebate Program rebates than it receives on the sale of certain covered outpatient drugs. Additionally, on July 9, 2021, President Biden issued an Executive Order to promote competition in the U.S. economy that included several initiatives addressing prescription drugs. Among other provisions, the Executive Order stated that the Biden administration will "support aggressive legislative reforms that would lower prescription drug prices, including by allowing Medicare to negotiate drug prices, by imposing inflation caps, and through other related reforms." In response to the Executive Order, on September 9, 2021, HHS issued a Comprehensive Plan for Addressing High Drug Prices that identified potential legislative policies and administrative tools that Congress and the agency can pursue in order to make drug prices more affordable and equitable, improve and promote competition throughout the prescription drug industry, and foster scientific innovation. Most recently, on May 10, 2022, the Biden Administration announced a plan to combat inflation that included a call to Congress to lower prescription drug costs and health premiums by allowing Medicare to negotiate drug prices, penalizing drug companies that raise prices faster than inflation, and making other needed reforms to lower drug prices. 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 replace 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. 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. Further, it is possible that additional governmental action is taken in response to the COVID-19 pandemic.

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 EU GDPR and/or the UK GDPR and other regulations. These laws may impact, among other things, our research, sales, marketing, education and patient assistance 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 anything of value as 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 and their business associates that perform certain services involving the use or disclosure of individually identifiable health information as well as their covered subcontractors, 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 HHS information related to payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists, and chiropractors), teaching hospitals, physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists, and certified nurse midwives, as well as ownership and investment interests held by physicians and their immediate family members and applicable group purchasing organizations;
- the Federal Food, Drug, and Cosmetic Act (FDCA), which, among other things, prohibits the adulteration and misbranding of drugs and biological products;
- 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 EU GDPR and/or the UK GDPR (together, GDPR) and other EU Member State or English data protection legislation, which require data controllers and processors to (i) implement administrative, physical, technical, and organizational safeguards to protect personal information; (ii) establish an appropriate and valid legal basis for processing personal information (iii) comply with accountability transparency requirements regarding the processing of personal information, which require controllers to demonstrate and record compliance with the GDPR and to provide more detailed information to data subjects regarding processing ; (iv) comply with data protection rights of data subjects including a right of access to and rectification of personal information, a right to obtain restriction of processing or to object to processing of personal information, a right to ask for a copy of personal information to be provided to a third party in a useable format and erasing personal information in certain circumstances; (v) report certain personal data breaches to the relevant supervisory authority without undue delay (and no later than 72 hours, where feasible); (vi) obtain explicit consent for collection of sensitive personal information such as health data; and (vii) consider data protection as any new products or services are developed and to limit the amount of personal information processed. In addition, the GDPR prohibits the international transfer of personal information outside of the EU and/or the UK including to the U.S., unless made to a country deemed to have adequate data privacy laws by the European Commission and/or the UK or a data transfer mechanism in accordance with the GDPR (as applicable) has been put in place.

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, corporate 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.

In June 2016, the electorate in the UK voted in favor of leaving the EU (commonly referred to as "Brexit"). Thereafter, in March 2017, the country formally notified the EU of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty and the UK formally left the EU on January 31, 2020. A transition period began on February 1, 2020, during which EU pharmaceutical law remained applicable to the UK. This transition period ended on December 31, 2020. The EU-UK Trade and Cooperation Agreement (TCA) was agreed on December 24, 2020. The UK and EU agreed that the TCA would apply provisionally from January 1, 2021, the TCA was ratified on April 30, 2021 and came into force on May 1, 2021.

Since the regulatory framework in the UK covering the quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from EU Directives and Regulations, Brexit could materially impact the future regulatory regime which applies to products and the approval of product candidates in the UK, as UK legislation now has the potential to diverge from EU legislation. It remains to be seen how Brexit will fully impact regulatory requirements for product candidates and products in the UK in the long-term.

At present, the regulatory framework for medicines that existed before the end of the transition period has effectively been preserved in UK domestic legislation as 'retained EU law' which has prevented substantial divergence to the regulation of medicines so far. However, the UK Government has now introduced the Retained EU Law (Revocation and Reform) Bill 2022 which intends to end the "special status" of retained EU law under UK law. If enacted into law, this would facilitate departure from retained EU law, and may lead to greater regulatory divergence between the EU and UK in the future.

Some changes to the UK legislation have been immediately necessary, including the implementation of the Northern Ireland Protocol (NIP), pursuant to which, the EU pharmaceutical legal framework acquis continues to apply in Northern Ireland (subject to periodic consent of the Northern Ireland Legislative Assembly), and only products compliant with EU law can be placed in the Northern Ireland market - adding an extra layer of regulatory complexity (although certain limited and temporary exceptions have been agreed between the EU and UK). The future of the NIP remains uncertain as the UK government has introduced the 'Northern Ireland Protocol Bill' which if enacted into law would enable the Government to unilaterally disapply parts of the NIP which may lead to changes to the regulatory environment in Northern Ireland, and may trigger retaliatory measures against the UK by the.

Companies now need to comply with a separate UK regulatory legal framework in order to commercialize medicinal products in Great Britain (namely, England, Wales and Scotland, as EU law continues to apply in Northern Ireland). For instance, Great Britain will no longer be covered by centralized marketing authorizations (under the Northern Irish Protocol, centralized marketing authorizations will continue to be recognized in Northern Ireland). All medicinal products with a current centralized marketing authorization were automatically converted to Great Britain marketing authorizations on January 1, 2021. For a period of three years from January 1, 2021, the MHRA may rely on a decision taken by the European Commission on the approval of a new marketing authorization in the centralized procedure, in order to more quickly grant a new Great Britain marketing authorization. A separate application will, however, still be required. The MHRA has also introduced new procedures aimed at accelerating regulatory approvals and time to market, including 'rolling reviews' and the Innovative Licensing and Access Pathway (ILAP) although eligibility requirements apply.

The TCA and the proposed Bills allow for future deviation from the current regulatory framework and it is not known if and/or when any deviations may occur, which may have an impact on development, manufacture, marketing authorization, commercial sales and distribution of pharmaceutical products.

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. Actions taken by the Russian Federation in Ukraine and surrounding areas have destabilized the region and caused the adoption of comprehensive sanctions by, among others, the EU, the United States

and the UK, which restrict a wide range of trade and financial dealings with Russia and Russian persons, as well as certain regions in Ukraine, including by imposing stricter export controls, prohibiting dealings with major Russian banks and credit institutions, and prohibiting trade with the Donetsk, Luhansk, Kherson and Zaporizhzhia regions of Ukraine. In addition, clinical site initiation and patient enrollment may be delayed, and we may not be able to access sites for initiation, monitoring and data collection in regions affected by the Russian invasion of Ukraine, including due to the prioritization of hospital resources away from clinical trials or as a result of government imposed curfews, warfare, violence or other governmental action or events that restrict movement. Some patients may not be able to comply with clinical trial protocols if the conflict impedes patient movement or interrupts healthcare services. We could also experience disruptions in our supply chain or limits to our ability to obtain sufficient materials for our drug products in certain regions. D-LIVR is a global Phase 3 trial that has enrolled 407 patients across 116 clinical trial sites in 22 countries, including five sites in Ukraine. While we believe that the study is more than adequately powered to demonstrate statistical significance over placebo even if patients from Ukraine discontinue from the study, impacts related to the Russian invasion of Ukraine may have a material adverse effect on our ability to adequately conduct the D-LIVR trial in the region. 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 operations and prospects.

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, other natural disasters, and 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 shelter-in-place orders 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 GCPs, with any material protocol deviation reviewed and approved by the site 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 and EMA may continue to suspend, prioritize or delay certain foreign inspections, and if there continues to be a suspension or delay in inspections, our product application reviews and potential approvals could be impacted or delayed. In response to the COVID-19 pandemic, on March 10, 2020 the FDA announced its intention to postpone most inspections of foreign manufacturing facilities. On March 18, 2020, the FDA announced its intention to temporarily postpone routine surveillance inspections of domestic manufacturing facilities and provided guidance regarding the conduct of clinical trials. Subsequently, on July 10, 2020, the FDA announced its intention to resume certain on-site inspections of domestic manufacturing facilities subject to a risk-based prioritization system. In May 2021, the FDA updated its guidance, first published in August 2020, clarifying how it intends to conduct inspections during the COVID-19 pandemic, including how it plans to determine which inspections are "mission critical." In November 2021, FDA provided an update to the May 2021 "Resiliency Roadmap for FDA Inspectional Oversight" noting completion of "mission critical" work over the previous year. In the update, FDA noted that it "is continuing to complete mission-critical work, prioritize other higher-tiered inspectional needs (e.g., for-cause inspections), and carry out surveillance inspections using risk-based approaches for evaluating public health impact." Further, ongoing surges in COVID-19 case numbers with the emergence of new variants and sub-variants have contributed to interruptions in FDA's surveillance capabilities. In light of high positivity rates and hospitalizations, FDA made temporary changes in late 2021, including temporarily postponing certain inspection activities from December 29, 2021 to January 19, 2022. On February 2, 2022, FDA announced that it would resume domestic surveillance inspections across all product areas on February 7, 2022. We cannot predict whether, and when, FDA will decide to pause or resume inspections due to the COVID-19 pandemic.

The FDA intends to use the roadmap as a risk-based assessment system to identify the categories of regulatory activity that can occur within a given geographic area, ranging from mission critical inspections to resumption of all regulatory activities. On-going surges, new variants, or additional factors may further affect inspection timelines.

Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic, including providing guidance regarding the conduct of clinical trials. If global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, or impact reviews or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

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 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, BLA, 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, NDA, or BLA (or the EMA’s review of that MAA);
- 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 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.

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 Columbia Threadneedle Investments, 683 Capital Management, BlackRock Institutional Trust, and The Vanguard Group, 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.

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. Under legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, as modified by the Coronavirus Aid, Relief, and Economic Security Act (CARES Act), U.S. federal net operating loss carryforwards generated in tax years beginning after December 31, 2017 may be carried forward indefinitely but, in the case of tax years beginning after December 31, 2020, may only be used to offset 80% of taxable income annually. In addition, California has enacted A.B. 85 which imposed limits on the usability of California state net operating losses and certain tax credits in tax years beginning after 2019 and before 2023. Such limitations could result in the expiration of portions of our net operating loss and tax credit carryforwards before utilization. On February 9, 2022, Senate Bill No. 113 was signed into California law and reinstates the net operating loss deduction, and removes the above-described temporary limitation on allowable credits, for taxable years beginning on or after January 1, 2022. 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 December 31, 2020. Based upon this assessment, we have experienced ownership changes on April 20, 2016, October 18, 2018 and December 31, 2020. 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

Exhibit Number	Description of Document
3.1	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).
3.2	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).
3.3	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).
3.4	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).
10.1	Loan and Security Agreement, dated June 1, 2022, among Eiger BioPharmaceuticals, Inc., its domestic subsidiaries and Innovatus Life Sciences Lending Fund I, LP, as collateral agent and lender (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K, originally filed with the SEC on June 7, 2022).
10.2	Common Stock Purchase Agreement, dated June 1, 2022, among Eiger BioPharmaceuticals, Inc., Innovatus Life Sciences Lending Fund I, LP, Innovatus Life Sciences Offshore Fund I-A, LP, Innovatus Flagship Fund I, LP, and Innovatus Flagship Offshore Fund I, LP (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K, originally filed with the SEC on June 7, 2022).
31.1**	Certification of Principal Executive Officer required by Rule 13a-14(a) or Rule 15d-14(a).
31.2**	Certification of Principal Financial Officer required by Rule 13a-14(a) or Rule 15d-14(a).
32.1+	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).
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, 2022, 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.

Eiger BioPharmaceuticals, Inc.

Date: November 3, 2022

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

Eiger BioPharmaceuticals, Inc.

Date: November 3, 2022

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

Exhibit 31.1

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 3, 2022

/s/ David A. Cory

David A. Cory
Chief Executive Officer

Exhibit 31.2

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 3, 2022

/s/ Sriram Ryali

Sriram Ryali
Chief Financial Officer

Exhibit 32.1

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, 2022, 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 3, 2022

/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."