

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

**FORM 10-Q**

(Mark One)

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

For the quarterly period ended September 30, 2020

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission file number: 001-35668

**INTERCEPT PHARMACEUTICALS, INC.**

(Exact Name of Registrant as Specified in Its Charter)

**Delaware**

(State or Other Jurisdiction of  
Incorporation or Organization)

**22-3868459**

(I.R.S. Employer  
Identification No.)

**10 Hudson Yards, 37<sup>th</sup> Floor**

**New York, NY 10001**

(Address of Principal Executive Offices and Zip Code)

**(646) 747-1000**

(Registrant's Telephone Number, Including Area Code)

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

<u>Title of each class</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
Common Stock, par value \$0.001 per share	ICPT	Nasdaq Global Select Market

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes  No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes  No

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

Large accelerated filer   
Non-accelerated filer

Accelerated filer   
Smaller reporting company   
Emerging growth company

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

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

The number of shares of the registrant's common stock outstanding as of September 30, 2020 was 32,992,436.

**Intercept Pharmaceuticals, Inc.**

**INDEX**

**PART I**  
**FINANCIAL INFORMATION**

<a href="#">Item 1.</a>	<a href="#">Financial Statements</a>	
	<a href="#">Condensed Consolidated Balance Sheets at September 30, 2020 (Unaudited) and December 31, 2019 (Audited)</a>	8
	<a href="#">Condensed Consolidated Statements of Operations for the three and nine-month periods ended September 30, 2020 and 2019 (Unaudited)</a>	9
	<a href="#">Condensed Consolidated Statements of Comprehensive Loss for the three and nine-month periods ended September 30, 2020 and 2019 (Unaudited)</a>	10
	<a href="#">Condensed Consolidated Statements of Changes in Stockholders' (Deficit) Equity for the three and nine-month periods ended September 30, 2020 and 2019 (Unaudited)</a>	11
	<a href="#">Condensed Consolidated Statements of Cash Flows for the nine-month periods ended September 30, 2020 and 2019 (Unaudited)</a>	13
	<a href="#">Notes to Condensed Consolidated Financial Statements (Unaudited)</a>	14
<a href="#">Item 2.</a>	<a href="#">Management's Discussion and Analysis of Financial Condition and Results of Operations</a>	33
<a href="#">Item 3.</a>	<a href="#">Quantitative and Qualitative Disclosures About Market Risk</a>	43
<a href="#">Item 4.</a>	<a href="#">Controls and Procedures</a>	44

**PART II**  
**OTHER INFORMATION**

<a href="#">Item 1.</a>	<a href="#">Legal Proceedings</a>	45
<a href="#">Item 1A.</a>	<a href="#">Risk Factors</a>	45
<a href="#">Item 2.</a>	<a href="#">Unregistered Sales of Equity Securities and Use of Proceeds</a>	100
<a href="#">Item 6.</a>	<a href="#">Exhibits</a>	101
	<a href="#">Exhibit Index</a>	102
	<a href="#">Signatures</a>	103

Unless the context otherwise requires, references in this Quarterly Report on Form 10-Q to “we,” “our,” “us” and the “Company” refer, collectively, to Intercept Pharmaceuticals, Inc., a Delaware corporation, and its consolidated subsidiaries.

## CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements, including, but not limited to, statements regarding the progress, timing and results of our clinical trials, including our clinical trials for the treatment of nonalcoholic steatohepatitis (“NASH”), the safety and efficacy of our approved product, Ocaliva (obeticholic acid or “OCA”) for primary biliary cholangitis (“PBC”), and our product candidates, including OCA for liver fibrosis due to NASH, the timing and acceptance of our regulatory filings and the potential approval of OCA for liver fibrosis due to NASH, the review of our New Drug Application for OCA for the treatment of liver fibrosis due to NASH by the U.S. Food and Drug Administration (the “FDA”), our intent to work with the FDA to address the issues raised in a complete response letter (“CRL”), the potential commercial success of OCA, as well as our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans and objectives.

These statements constitute 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 words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “possible,” “continue” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of their dates, and we undertake no obligation to update any forward-looking statement except as required by law. These forward-looking statements are based on estimates and assumptions by our management that, although believed to be reasonable, are inherently uncertain and subject to a number of risks.

The following represent some, but not necessarily all, of the factors that could cause actual results to differ materially from historical results or those anticipated or predicted by our forward-looking statements:

- our ability to successfully commercialize Ocaliva for PBC;
- our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization;
- our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL by the FDA; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; or any future determination that the regulatory applications and subsequent information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval;
- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of any of our products or product candidates;
- any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions, or otherwise limit the sale of such product or product candidate;
- the initiation, timing, cost, conduct, progress and results of our research and development activities, preclinical studies and clinical trials, including any issues, delays or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials;

## [Table of Contents](#)

- our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our clinical trial activities;
- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to NASH, if approved;
- our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend and enforce any patent claims or other intellectual property rights;
- the size and growth of the markets for our products and product candidates and our ability to serve those markets;
- the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH or our other product candidates among physicians, patients and healthcare payors;
- the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products;
- our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties;
- competition from existing drugs or new drugs that become available;
- our ability to prevent system failures, data breaches or violations of data protection laws;
- costs and outcomes relating to any disputes, governmental inquiries or investigations, regulatory proceedings, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation;
- our collaborators' election to pursue research, development and commercialization activities;
- our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise;
- our need for and ability to generate or obtain additional financing;
- our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof;
- our use of cash and short-term investments;
- our ability to acquire, license and invest in businesses, technologies, product candidates and products;
- our ability to attract and retain key personnel to manage our business effectively;
- our ability to manage the growth of our operations, infrastructure, personnel, systems and controls;
- our ability to obtain and maintain adequate insurance coverage;

- the impact of COVID-19, including any impact on our results of operations or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof;
- the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and
- the other risks and uncertainties identified under the captions “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Quarterly Report on Form 10-Q and in our other periodic filings filed with the U.S. Securities and Exchange Commission, including our Annual Report on Form 10-K for the year ended December 31, 2019.

#### **NOTE REGARDING TRADEMARKS**

The Intercept Pharmaceuticals<sup>®</sup> name and logo and the Ocaliva<sup>®</sup> name and logo are either registered or unregistered trademarks or trade names of the Company in the United States and/or other countries. All other trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, trademarks and trade names referred to in this Quarterly Report on Form 10-Q may appear without the <sup>®</sup> and <sup>™</sup> symbols, but those references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or that the applicable owner will not assert its rights to these trademarks and trade names.

## SUMMARY RISK FACTORS

Investing in our securities involves a high degree of risk. Investors should carefully consider the risks and uncertainties discussed under the caption “Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2019 before deciding whether to invest in our securities. The following is a list of some of these risks:

### ***Risks Related to Our Financial Position and Need for Additional Capital***

- We are currently dependent on the successful commercialization of Ocaliva for PBC. To the extent Ocaliva is not commercially successful, our business, financial condition and results of operations may be materially and adversely affected and the price of our common stock may decline.
- We have never been profitable. We expect to incur losses for the foreseeable future, and we may never achieve or sustain profitability.
- We will require substantial additional funding, which may not be available to us on acceptable terms, if at all. If adequate funds are not available to us, we may be required to delay, limit, reduce or cease our operations.
- Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.
- We have a limited operating history as a commercial organization, which may make it difficult to predict our future performance, and we expect to continue to face a number of factors that may cause operating results to fluctuate.

### ***Risks Related to the Development and the Regulatory Review and Approval of Our Products and Product Candidates***

- We cannot be certain whether Ocaliva will receive full approval for PBC in jurisdictions where it has previously received accelerated or conditional approval, or that Ocaliva will be approved for PBC in any jurisdictions beyond those in which it is currently approved. Furthermore, OCA may not be approved on an accelerated basis, or at all, for NASH or any other indication beyond PBC and we may not receive regulatory approval for any other product candidate. Without regulatory approval, we will not be able to market and commercialize our product candidates.
- We are developing product candidates for the treatment of rare diseases or diseases for which there are no or limited therapies, such as PBC and NASH, and for some of which there is little clinical experience, and our development approach involves new endpoints and methodologies. As a result, there is a heightened risk that we will not be able to gain agreement with regulatory authorities regarding an acceptable development plan, that the outcome of our clinical trials will not be favorable or that, even if favorable, regulatory authorities may not find the results of our clinical trials to be sufficient for marketing approval.
- Delays or difficulties in the commencement, enrollment and completion of our clinical trials and studies could increase our product development costs and delay, limit or prevent us from obtaining regulatory approval for OCA and our other product candidates.
- COVID-19 could materially and adversely affect our clinical trials.
- Failure can occur at any stage of clinical development. The results of earlier clinical trials are not necessarily predictive of future results and any product candidate we or our collaborators advance through clinical trials, including OCA, may not have favorable results in later clinical trials or receive or maintain regulatory approval.
- Our product candidates may have undesirable side effects which may delay or prevent marketing approval, or, if approval is received, require that our products be taken off the market or include new or additional safety warnings. Any such events may limit our existing and future product sales and materially and adversely affect our business, financial condition and results of operations.
- We may not be able to obtain or, if approved, maintain orphan drug exclusivity for our approved products or product candidates, which could cause our revenues to suffer.

***Risks Related to the Commercialization of Our Products***

- Sales of Ocaliva may be adversely affected by safety and labeling changes required by the FDA.
- We are subject to uncertainty relating to pricing and reimbursement. Failure to obtain or maintain adequate coverage, pricing and reimbursement for Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, or our other future approved products, if any, could have a material adverse impact on our ability to commercialize such products.
- Legislative and regulatory healthcare reform may adversely affect our business.
- Ocaliva and our other future approved products, if any, may not achieve broad market acceptance among physicians, patients and healthcare payors, and revenues generated from their sales may be limited as a result.
- If we fail to develop OCA for additional indications such as NASH, our commercial opportunity will be limited.

***Risks Related to Our Business and Strategy***

- We depend on third-party contractors for a substantial portion of our operations and may not be able to control their work as effectively as if we performed these functions ourselves.
- We face rapid technological change and competition from other biotechnology and pharmaceutical companies. Our operating results will suffer if we fail to compete effectively.
- Our business and operations would suffer in the event of system failures, data breaches or violations of data protection laws.

***Risks Related to Our Intellectual Property***

- It is difficult and costly to protect our proprietary rights, and we may not be able to ensure their protection. If our patent position does not adequately protect our products such as Ocaliva and product candidates such as OCA for liver fibrosis due to NASH, others may compete against us more directly, which could harm our business, possibly materially.
- If we do not obtain protection under the Hatch-Waxman Act in the United States (and similar legislation outside of the United States) extending the terms of our patents and/or providing data or other exclusivity for our products and product candidates, our business may be materially harmed.
- We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights, and such litigation may divert the attention of our management and scientific personnel and adversely affect our development and commercialization efforts.

***Risks Related to Our Indebtedness***

- Servicing our debt will require significant amounts of cash, and we may not have sufficient cash flow from our business to effectively service our debt.
- We may incur substantially more debt or take other actions that would affect our ability to pay the principal of and interest on our debt.

***Risks Related to Ownership of Our Common Stock***

- We have previously been, and are currently, subject to securities class action litigation and may be subject to similar or other litigation in the future. Such matters can be expensive, time-consuming and have a material adverse effect on our business, results of operations and financial condition.
- Our stock price has been and may in the future be volatile, which could cause holders of our common stock to incur substantial losses.

## PART I

## Item 1. Financial Statements.

**INTERCEPT PHARMACEUTICALS, INC.**  
**Condensed Consolidated Balance Sheets**  
(In thousands, except share and per share data)

	September 30, 2020 (Unaudited)	December 31, 2019 (Audited)
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 45,311	\$ 70,055
Restricted cash	6,180	4,725
Investment debt securities, available-for-sale	445,269	582,567
Accounts receivable, net of allowance for credit losses of \$224 and \$0, respectively	39,049	38,044
Prepaid expenses and other current assets	25,282	25,924
Total current assets	561,091	721,315
Fixed assets, net	4,898	5,202
Inventory	8,359	8,462
Security deposits	6,949	6,661
Other assets	10,055	13,246
Total assets	<u>\$ 591,352</u>	<u>\$ 754,886</u>
<b>Liabilities and Stockholders' (Deficit) Equity</b>		
Current liabilities:		
Accounts payable, accrued expenses and other liabilities	\$ 157,674	\$ 153,968
Short-term interest payable	5,450	8,037
Total current liabilities	163,124	162,005
Long-term liabilities:		
Long-term debt	553,216	532,078
Long-term other liabilities	5,299	9,247
Total liabilities	<u>\$ 721,639</u>	<u>\$ 703,330</u>
Commitments and contingencies (Note 18)		
Stockholders' (deficit) equity:		
Common stock par value \$0.001 per share; 90,000,000 and 45,000,000 shares authorized; 32,992,436 and 32,853,066 shares issued and outstanding as of September 30, 2020 and December 31, 2019, respectively	33	33
Additional paid-in capital	2,217,625	2,176,133
Accumulated other comprehensive loss, net	(1,746)	(1,144)
Accumulated deficit	(2,346,199)	(2,123,466)
Total stockholders' (deficit) equity	<u>(130,287)</u>	<u>51,556</u>
Total liabilities and stockholders' (deficit) equity	<u>\$ 591,352</u>	<u>\$ 754,886</u>

See accompanying notes to the condensed consolidated financial statements.

**INTERCEPT PHARMACEUTICALS, INC.**  
**Condensed Consolidated Statements of Operations**  
**(Unaudited)**  
**(In thousands, except per share data)**

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
<b>Revenue:</b>				
Product revenue, net	\$ 79,521	\$ 61,545	\$ 229,422	\$ 179,286
Licensing revenue	—	405	—	1,216
Total revenue	<u>79,521</u>	<u>61,950</u>	<u>229,422</u>	<u>180,502</u>
<b>Operating expenses:</b>				
Cost of sales	1,826	487	4,555	1,738
Selling, general and administrative	70,619	76,828	262,537	223,738
Research and development	48,858	60,168	139,587	178,163
Restructuring	13,381	—	13,381	—
Total operating expenses	<u>134,684</u>	<u>137,483</u>	<u>420,060</u>	<u>403,639</u>
Operating loss	<u>(55,163)</u>	<u>(75,533)</u>	<u>(190,638)</u>	<u>(223,137)</u>
<b>Other income (expense):</b>				
Interest expense	(12,091)	(11,795)	(35,801)	(29,518)
Other income, net	785	2,495	3,706	6,132
Total other (expense), net	<u>(11,306)</u>	<u>(9,300)</u>	<u>(32,095)</u>	<u>(23,386)</u>
Net loss	<u>\$ (66,469)</u>	<u>\$ (84,833)</u>	<u>\$ (222,733)</u>	<u>\$ (246,523)</u>
<b>Net loss per common and potential common share:</b>				
Basic and diluted	\$ (2.01)	\$ (2.59)	\$ (6.76)	\$ (7.88)
<b>Weighted average common and potential common shares outstanding:</b>				
Basic and diluted	32,989	32,717	32,957	31,275

See accompanying notes to the condensed consolidated financial statements.

**INTERCEPT PHARMACEUTICALS, INC.**  
**Condensed Consolidated Statements of Comprehensive Loss**  
**(Unaudited)**  
**(In thousands)**

	<u>Three Months Ended</u>		<u>Nine Months Ended</u>	
	<u>September 30,</u>		<u>September 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
Net loss	\$ (66,469)	\$ (84,833)	\$ (222,733)	\$ (246,523)
Other comprehensive (loss) income:				
Net changes related to available-for-sale investment debt securities:				
Unrealized (losses) gains on investment debt securities	(730)	451	230	1,646
Reclassification adjustment for realized gains on investment debt securities included in other income, net	—	2	53	6
Net unrealized (losses) gains on investment debt securities	\$ (730)	\$ 453	\$ 283	\$ 1,652
Foreign currency translation gains (losses)	109	(687)	(891)	(463)
Other comprehensive (loss) income	\$ (621)	\$ (234)	\$ (608)	\$ 1,189
Comprehensive loss	<u>\$ (67,090)</u>	<u>\$ (85,067)</u>	<u>\$ (223,341)</u>	<u>\$ (245,334)</u>

See accompanying notes to the condensed consolidated financial statements.

**INTERCEPT PHARMACEUTICALS, INC.**  
**Condensed Consolidated Statements of Changes in Stockholders' (Deficit) Equity**  
**(Unaudited)**  
**(In thousands)**

Three months ended September 30, 2020

	<u>Common Stock</u>		<u>Additional Paid-in Capital</u>	<u>Accumulated Other Comprehensive</u>	<u>Accumulated</u>	<u>Total</u>
	<u>Shares</u>	<u>Amount</u>		<u>Loss, Net</u>	<u>Deficit</u>	<u>Stockholders' Deficit</u>
Balance - June 30, 2020	32,981	\$ 33	\$ 2,202,026	\$ (1,125)	\$ (2,279,730)	\$ (78,796)
Stock-based compensation	—	—	15,825	—	—	15,825
Net proceeds from exercise of stock options	14	—	—	—	—	—
Employee withholding taxes related to stock-based awards	(3)	—	(226)	—	—	(226)
Other comprehensive loss	—	—	—	(621)	—	(621)
Net loss	—	—	—	—	(66,469)	(66,469)
Balance - September 30, 2020	<u>32,992</u>	<u>\$ 33</u>	<u>\$ 2,217,625</u>	<u>\$ (1,746)</u>	<u>\$ (2,346,199)</u>	<u>\$ (130,287)</u>

Nine months ended September 30, 2020

	<u>Common Stock</u>		<u>Additional Paid-in Capital</u>	<u>Accumulated Other Comprehensive</u>	<u>Accumulated</u>	<u>Total</u>
	<u>Shares</u>	<u>Amount</u>		<u>Loss, Net</u>	<u>Deficit</u>	<u>Stockholders' Equity (Deficit)</u>
Balance - December 31, 2019	32,853	\$ 33	\$ 2,176,133	\$ (1,144)	\$ (2,123,466)	\$ 51,556
Stock-based compensation	—	—	44,381	—	—	44,381
Net proceeds from exercise of stock options	149	—	(1,052)	—	—	(1,052)
Employee withholding taxes related to stock-based awards	(10)	—	(1,837)	—	—	(1,837)
Other comprehensive loss	—	—	—	(602)	—	(602)
Net loss	—	—	—	—	(222,733)	(222,733)
Balance - September 30, 2020	<u>32,992</u>	<u>\$ 33</u>	<u>\$ 2,217,625</u>	<u>\$ (1,746)</u>	<u>\$ (2,346,199)</u>	<u>\$ (130,287)</u>

**Three months ended September 30, 2019**

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss, Net	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Balance - June 30, 2019	32,696	\$ 33	\$ 2,116,481	\$ (836)	\$ (1,940,475)
Stock-based compensation	—	—	13,130	—	—	13,130
Recognition of debt discount on 2026 Convertible Notes	—	—	26,577	—	—	26,577
Net proceeds from exercise of stock options	37	—	773	—	—	773
Employee withholding taxes related to stock-based awards	—	—	119	—	—	119
Other comprehensive loss	—	—	—	(234)	—	(234)
Net loss	—	—	—	—	(84,833)	(84,833)
Balance - September 30, 2019	<u>32,733</u>	<u>\$ 33</u>	<u>\$ 2,157,080</u>	<u>\$ (1,070)</u>	<u>\$ (2,025,308)</u>	<u>\$ 130,735</u>

**Nine months ended September 30, 2019**

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss, Net	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Balance - December 31, 2018	29,694	\$ 30	\$ 1,800,144	\$ (2,259)	\$ (1,778,785)
Stock-based compensation	—	—	42,809	—	—	42,809
Recognition of debt discount on 2026 Convertible Notes	—	—	85,915	—	—	85,915
Issuance of common stock from public and private placement offerings, net of underwriting fees and issuance costs	2,880	3	227,177	—	—	227,180
Net proceeds from exercise of stock options	159	—	2,511	—	—	2,511
Employee withholding taxes related to stock-based awards	—	—	(1,476)	—	—	(1,476)
Other comprehensive income	—	—	—	1,189	—	1,189
Net loss	—	—	—	—	(246,523)	(246,523)
Balance - September 30, 2019	<u>32,733</u>	<u>\$ 33</u>	<u>\$ 2,157,080</u>	<u>\$ (1,070)</u>	<u>\$ (2,025,308)</u>	<u>\$ 130,735</u>

See accompanying notes to the condensed consolidated financial statements.

**INTERCEPT PHARMACEUTICALS, INC.**  
**Condensed Consolidated Statements of Cash Flows**  
**(Unaudited)**  
**(In thousands)**

	<b>Nine Months Ended September 30,</b>	
	<b>2020</b>	<b>2019</b>
<b>Cash flows from operating activities:</b>		
Net loss	\$ (222,733)	\$ (246,523)
<b>Adjustments to reconcile net loss to net cash used in operating activities:</b>		
Stock-based compensation	44,381	42,809
(Accretion) amortization of (discount) premium on investment debt securities	2,617	(595)
Amortization of deferred financing costs	1,883	1,529
Depreciation	2,282	2,839
Non-cash operating lease cost	4,645	3,974
Gain on lease termination	—	(1,995)
Loss on the disposal of fixed assets	—	2,682
Accretion of debt discount	19,255	15,051
Provision for allowance of credit losses	224	—
<b>Changes in operating assets:</b>		
Accounts receivable	(821)	(7,128)
Prepaid expenses and other current assets	(1,705)	(3,320)
Inventory	(110)	(2,240)
Security deposits	(263)	1,018
Other assets	—	(19,489)
<b>Changes in operating liabilities:</b>		
Accounts payable, accrued expenses and other current liabilities	4,261	33,990
Operating lease liabilities	(5,380)	(5,019)
Interest payable	(2,587)	(2,012)
Deferred revenue	—	(1,216)
Long-term other liabilities	—	9,516
Net cash (used in) operating activities	<u>(154,051)</u>	<u>(176,129)</u>
<b>Cash flows from investing activities:</b>		
Purchases of investment debt securities	(282,629)	(560,733)
Sales and maturities of investment debt securities	417,592	306,898
Purchases of equipment, leasehold improvements, and furniture and fixtures	(1,990)	(1,041)
Net cash provided by (used in) investing activities	<u>132,973</u>	<u>(254,876)</u>
<b>Cash flows from financing activities:</b>		
Proceeds from issuance of 2026 Convertible Notes, net of issuance costs	—	223,424
Proceeds from issuance of common stock, net of issuance costs	—	227,180
Proceeds from exercise of options, net	1,300	2,511
Payments of employee withholding taxes related to stock-based awards	(1,837)	(1,476)
Net cash (used in) provided by financing activities	<u>(537)</u>	<u>451,639</u>
Effect of exchange rate changes on cash, cash equivalents and restricted cash	<u>(1,674)</u>	<u>(505)</u>
Net (decrease) increase in cash, cash equivalents and restricted cash	<u>(23,289)</u>	<u>20,129</u>
Cash, cash equivalents and restricted cash at beginning of period	74,780	43,248
Cash, cash equivalents and restricted cash at end of period	<u>\$ 51,491</u>	<u>\$ 63,377</u>
<b>Supplemental disclosure of non-cash transactions:</b>		
Right-of-use asset obtained in exchange for new operating lease obligations	<u>\$ 1,230</u>	<u>\$ —</u>
<b>Reconciliation of cash, cash equivalents and restricted cash included in the condensed consolidated balance sheets:</b>		
Cash and cash equivalents	\$ 45,311	\$ 63,377
Restricted cash	6,180	—
Total cash, cash equivalents and restricted cash	<u>\$ 51,491</u>	<u>\$ 63,377</u>

See accompanying notes to the condensed consolidated financial statements.

**INTERCEPT PHARMACEUTICALS, INC.**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

**1. Overview of Business**

Intercept Pharmaceuticals, Inc. (the “Company”) is a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, including primary biliary cholangitis (“PBC”) and nonalcoholic steatohepatitis (“NASH”). The Company currently has one marketed product, Ocaliva (obeticholic acid or “OCA”). Founded in 2002 in New York, the Company has operations in the United States, Europe and Canada.

**2. Basis of Presentation**

The Company’s financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (“U.S. GAAP”). All intercompany balances and transactions have been eliminated in consolidation. Certain information that is normally required by U.S. GAAP has been condensed or omitted in accordance with rules and regulations of the U.S. Securities and Exchange Commission (“SEC”). Operating results for the three and nine months ended September 30, 2020 are not necessarily indicative of the results that may be expected for any future period or for the year ending December 31, 2020. In the opinion of management, these unaudited condensed consolidated financial statements include all normal and recurring adjustments considered necessary for a fair presentation of these interim unaudited condensed consolidated financial statements.

These unaudited condensed consolidated financial statements should be read in conjunction with the Company’s audited consolidated financial statements and the notes thereto for the year ended December 31, 2019, included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2019 filed with the SEC.

***Use of Estimates***

The preparation of these unaudited condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and judgments that affect the reported amounts of assets and liabilities, the disclosure of assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results may differ from these estimates.

We are not presently aware of any events or circumstances arising from the coronavirus (“COVID-19”) pandemic that would require us to update our estimates, judgments or revise the carrying value of our assets or liabilities.

**3. Summary of Significant Accounting Policies**

With the exception of the change for the accounting of credit losses as a result of the adoption of Accounting Standards Update (“ASU”) No. 2016-13, “Financial Instruments-Credit Losses: Measurement of Credit Losses on Financial Instruments” and related amendments (collectively, “ASC 326”), there have been no new or material changes to the significant accounting policies discussed in the Company’s Annual Report on Form 10-K for the year ended December 31, 2019.

***Credit Losses***

***Accounts receivable***

The allowance for credit losses is based on the Company’s assessment of the collectibility of customer accounts. The Company regularly reviews the allowance by considering factors such as historical experience, the aging of the accounts receivable balances, credit conditions that may affect a customer’s ability to pay, current and forecast economic conditions and other relevant factors.

[Table of Contents](#)

The following table summarizes the allowance for credit losses activity on the Company's trade receivables for the nine-month period ended September 30, 2020 (in thousands):

Balance at December 31, 2019	\$	—
Provision for credit losses		235
Write-offs		(11)
Balance at September 30, 2020	\$	224

#### ***Available-for-sale investment debt securities***

For available-for-sale investment debt securities in an unrealized loss position, the Company first assesses whether it intends to sell the security or it is more likely than not that it will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the amortized cost basis is written down to fair value through income. For any investment debt securities that do not meet the criteria, the Company evaluates whether the decline in fair value has resulted from credit losses or other factors. Management considers the extent in which the fair value of the security is less than amortized costs, any changes to the rating of the security by a rating agency, changes in interest rates, and any other adverse factors related to the security. If the assessment indicates a credit loss, the present value of cash flows expected to be collected are compared to the amortized cost basis of the security. If the expected present value of cash flows is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded, limited to the amount that the fair value is below the amortized cost basis. Any impairment not recorded through an allowance is recognized in Other comprehensive (loss) income.

Changes in the allowance for credit losses are recorded as a provision for (or reversal of) credit loss expense. Losses are charged against the allowance when management believes the uncollectibility of the security is confirmed or whether either of the criteria regarding intent or requirement to sell is met.

The Company excludes accrued interest from both the fair value and amortized cost basis in the assessment of credit losses on its available-for-sale investment debt securities and will instead elect to write-off any uncollectible accrued interest receivable balances in a timely manner, which is defined by the Company as when interest due becomes 90 days delinquent.

#### ***Recent Accounting Pronouncements***

##### ***Recently Adopted Accounting Pronouncements***

In June 2016, the Financial Accounting Standards Board ("FASB") issued ASU No. 2016-13, which replaces the incurred loss impairment methodology under current U.S. GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. ASU 2016-13 was subsequently updated by ASU No. 2019-04, "Codification Improvements to Topic 326, Financial Instruments—Credit Losses, Topic 815, Derivatives and Hedging, and Topic 825, Financial Instruments", to clarify that entities should include recoveries when estimating the allowance for credit losses. The Company will be required to use a forward-looking expected credit loss model for accounts receivables, loans and other financial instruments. Credit losses relating to available-for-sale investment debt securities will also be recorded through an allowance for credit losses rather than as a reduction in the amortized cost basis of the securities. The Company adopted the practical expedient to exclude the accrued interest included in the fair value and amortized cost basis in the assessment of credit losses on its available-for-sale investment debt securities. This guidance is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019 and must be adopted using a modified retrospective approach, with certain exceptions. The Company adopted ASC 326 on January 1, 2020 and its adoption did not have a material impact on the Company's condensed consolidated financial statements and related disclosures.

In August 2018, the FASB issued ASU No. 2018-13, "Fair Value Measurement (Topic 820): Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement" ("ASU 2018-13"), which makes a number of changes meant to add, modify or remove certain disclosure requirements associated with the movement amongst or

hierarchy associated with Level 1, Level 2 and Level 3 fair value measurements. This guidance is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. Early adoption is permitted upon issuance of the update. The Company adopted ASU 2018-13 on January 1, 2020 and its adoption did not have an impact on the Company's condensed consolidated financial statements and related disclosures.

**Recent Accounting Pronouncements to be Adopted**

In December 2019, the FASB issued ASU No. 2019-12, "Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes ("ASU 2019-12"), which is intended to simplify various aspects related to accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in Topic 740 and also clarifies and amends existing guidance to improve consistent application. This guidance is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2020, with early adoption permitted. The Company is currently evaluating the impact of this standard on its consolidated financial statements and related disclosures.

In August 2020, the FASB issued ASU No. 2020-06, "Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40): Accounting for Convertible Instruments and Contracts in an Entity's Own Equity ("ASU 2020-06"), which simplifies accounting for convertible instruments by removing major separation models required under current U.S. GAAP. The ASU removes certain settlement conditions that are required for equity contracts to qualify for the derivative scope exception and it also simplifies the diluted earnings per share calculation in certain areas. Either a modified retrospective method of transition or a fully retrospective method of transition is permissible for the adoption of this standard. This guidance is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2021, with early adoption permitted. The Company plans to adopt the standard effective January 1, 2021 and is currently evaluating the impact of this standard on its consolidated financial statements and related disclosures.

**4. Cash, Cash Equivalents and Investment Debt Securities**

The following table summarizes the Company's cash, cash equivalents and investment debt securities as of September 30, 2020 and December 31, 2019:

	As of September 30, 2020				Fair Value
	Amortized Cost	Allowance for Credit Losses	Gross Unrealized Gains (in thousands)	Gross Unrealized Losses	
<b>Cash and cash equivalents:</b>					
Cash and money market funds	\$ 45,311	\$ —	\$ —	\$ —	\$ 45,311
Total cash and cash equivalents	45,311	—	—	—	45,311
<b>Investment debt securities:</b>					
Commercial paper	26,463	—	8	(1)	26,470
Corporate debt securities	417,727	—	1,159	(87)	418,799
Total investment debt securities	444,190	—	1,167	(88)	445,269
Total cash, cash equivalents and investment debt securities	\$ 489,501	\$ —	\$ 1,167	\$ (88)	\$ 490,580

	As of December 31, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
<b>Cash and cash equivalents:</b>				
Cash and money market funds	\$ 62,557	\$ —	\$ —	\$ 62,557
Commercial paper	7,498	—	—	7,498
Total cash and cash equivalents	70,055	—	—	70,055
<b>Investment debt securities:</b>				
Commercial paper	42,806	43	(1)	42,848
Corporate debt securities	538,965	835	(81)	539,719
Total investment debt securities	581,771	878	(82)	582,567
Total cash, cash equivalents and investment debt securities	\$ 651,826	\$ 878	\$ (82)	\$ 652,622

The aggregate fair value of the Company's available-for-sale investment debt securities that have been in a continuous unrealized loss position for less than twelve months or twelve months or longer is as follows:

	As of September 30, 2020					
	Less than 12 months		12 months or longer		Total	
	Fair Value	Gross Unrealized Losses	Fair Value	Gross Unrealized Losses	Fair Value	Gross Unrealized Losses
	(in thousands)					
Commercial paper	\$ 6,990	\$ (1)	\$ —	\$ —	\$ 6,990	\$ (1)
Corporate debt securities	108,502	(87)	—	—	108,502	(87)
Total	\$ 115,492	\$ (88)	\$ —	\$ —	\$ 115,492	\$ (88)

  

	As of December 31, 2019					
	Less than 12 months		12 months or longer		Total	
	Fair Value	Gross Unrealized Losses	Fair Value	Gross Unrealized Losses	Fair Value	Gross Unrealized Losses
	(in thousands)					
Commercial paper	\$ 11,976	\$ (1)	\$ —	\$ —	\$ 11,976	\$ (1)
Corporate debt securities	121,684	(81)	—	—	121,684	(81)
Total	\$ 133,660	\$ (82)	\$ —	\$ —	\$ 133,660	\$ (82)

At September 30, 2020, the Company had 43 available-for-sale investment debt securities in an unrealized loss position without an allowance for credit losses. Unrealized losses on corporate debt securities have not been recognized into income because the issuers' bonds are of high credit quality (rated A3/A- or higher), management does not intend to sell and it is likely that management will not be required to sell the securities prior to their anticipated recovery and the decline in fair value is largely due to market conditions and/or changes in interest rates. The issuers continue to make timely interest payments on the bonds. The fair value is expected to recover as the bonds approach maturity.

Accrued interest receivable on available-for-sale investment debt securities totaled \$2.3 million at September 30, 2020, is excluded from the estimate of credit losses and is included in Prepaid expenses and other current assets.

## 5. Fair Value Measurements

The carrying amounts of the Company's receivables and payables approximate their fair value due to their short maturities.

[Table of Contents](#)

Accounting principles provide guidance for using fair value to measure assets and liabilities. The guidance includes a three-level hierarchy of valuation techniques used to measure fair value, defined as follows:

- Unadjusted Quoted Prices — The fair value of an asset or liability is based on unadjusted quoted prices in active markets for identical assets or liabilities (Level 1).
- Pricing Models with Significant Observable Inputs — The fair value of an asset or liability is based on information derived from either an active market quoted price, which may require further adjustment based on the attributes of the financial asset or liability being measured, or an inactive market transaction (Level 2).
- Pricing Models with Significant Unobservable Inputs — The fair value of an asset or liability is primarily based on internally derived assumptions surrounding the timing and amount of expected cash flows for the financial instrument. Therefore, these assumptions are unobservable in either an active or inactive market (Level 3).

The Company considers an active market as one in which transactions for the asset or liability occur with sufficient frequency and volume to provide pricing information on an ongoing basis. Conversely, the Company views an inactive market as one in which there are few transactions for the asset or liability, the prices are not current, or price quotations vary substantially either over time or among market makers. Where appropriate, non-performance risk, or that of a counterparty, is considered in determining the fair values of liabilities and assets, respectively.

The Company's money market funds are classified within Level 1 of the fair value hierarchy because they are valued using quoted prices in active markets. Investment debt securities are classified as Level 2 instruments based on market pricing and other observable inputs.

Financial assets carried at fair value are classified in the tables below in one of the three categories described above:

	<u>Total</u>	<u>Fair Value Measurements Using</u>		
		<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>
		<u>(in thousands)</u>		
<b>September 30, 2020</b>				
<b>Assets</b>				
Cash and cash equivalents:				
Money market funds	\$ 22,234	\$ 22,234	\$ —	\$ —
Available-for-sale investment debt securities:				
Commercial paper	26,470	—	26,470	—
Corporate debt securities	418,799	—	418,799	—
Total financial assets	<u>\$ 467,503</u>	<u>\$ 22,234</u>	<u>\$ 445,269</u>	<u>\$ —</u>
<b>December 31, 2019</b>				
<b>Assets</b>				
Cash and cash equivalents:				
Money market funds	\$ 19,376	\$ 19,376	\$ —	\$ —
Commercial paper	7,498	—	7,498	—
Available-for-sale investment debt securities:				
Commercial paper	42,848	—	42,848	—
Corporate debt securities	539,719	—	539,719	—
Total financial assets	<u>\$ 609,441</u>	<u>\$ 19,376</u>	<u>\$ 590,065</u>	<u>\$ —</u>

[Table of Contents](#)

The aggregate fair value of all available-for-sale investment debt securities (commercial paper and corporate debt securities), by contractual maturity, are as follows:

	Fair Value as of	
	September 30, 2020	December 31, 2019
	(in thousands)	
Due in one year or less	\$ 340,022	\$ 473,602
Due after one year through two years	105,247	116,463
Total investment debt securities	<u>\$ 445,269</u>	<u>\$ 590,065</u>

Actual maturities may differ from contractual maturities because issuers may have the right to call or prepay obligations without call or prepayment penalties.

## 6. Fixed Assets, Net

Fixed assets are stated at cost and depreciated or amortized using the straight-line method based on useful lives as follows:

	Useful lives (Years)	September 30, 2020	December 31, 2019
		(in thousands)	
Office equipment and software	3	\$ 4,626	\$ 4,386
Leasehold improvements	Shorter of remaining lease term or useful life	12,017	10,489
Furniture and fixtures	7	4,208	4,032
Subtotal		20,851	18,907
Less: accumulated depreciation		(15,953)	(13,705)
Fixed assets, net		<u>\$ 4,898</u>	<u>\$ 5,202</u>

## 7. Inventory

Inventories are stated at the lower of cost or market. Inventories consisted of the following:

	September 30, 2020	December 31, 2019
	(in thousands)	
Work-in-process	\$ 7,842	\$ 8,302
Finished goods	517	160
Inventory	<u>\$ 8,359</u>	<u>\$ 8,462</u>

## 8. Leases

The Company leases various office spaces under non-cancelable operating leases with original lease periods expiring between the fourth quarter of 2020 and 2024. The Company also enters into leases for equipment. A number of the Company's leases include one or more options to renew, with renewal terms that can extend the lease term. The exercise of lease renewal options is typically at the sole discretion of the Company; therefore, all renewals to extend the lease terms are not included in the right-of-use ("ROU") assets and lease liabilities as they are not reasonably certain of exercise. The Company regularly evaluates the renewal options and when they are reasonably certain of exercise, includes the renewal period in the lease term. These operating leases do not contain material variable rent payments, residual value guarantees, covenants, or other restrictions.

The Company has elected the practical expedient to exclude short-term leases from its ROU assets and lease liabilities; therefore leases with an initial term of 12 months or less are not recorded on the balance sheet. The Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company elected the practical expedient not to separate non-lease components from all leases. As the Company's leases do not provide an implicit rate, the Company

[Table of Contents](#)

uses an incremental borrowing rate based on the information available at the lease commencement date in determining the present value of the lease payments. The Company's incremental borrowing rate is the estimated rate that would be required to pay for a collateralized borrowing equal to the total lease payment over the lease term. The Company estimates its incremental borrowing rate based on an analysis of publicly traded debt securities of companies with credit and financial profiles similar to its own adjusted to be commensurate with the term of the underlying lease and the Company's secured borrowing rate.

Operating lease assets and liabilities are classified on the condensed consolidated balance sheets as follows:

Leases	Classification	September 30, 2020	December 31, 2019
<b>Assets</b>			
(in thousands)			
Operating lease assets	Other assets	\$ 10,055	\$ 13,246
Total leased assets		<u>\$ 10,055</u>	<u>\$ 13,246</u>
<b>Liabilities</b>			
Current			
Operating lease liabilities	Accounts payable, accrued expenses and other liabilities	\$ 6,133	\$ 6,456
Noncurrent			
Operating lease liabilities	Long-term other liabilities	5,299	9,222
Total operating lease liabilities		<u>\$ 11,432</u>	<u>\$ 15,678</u>

Operating lease costs for the three and nine-month periods ended September 30, 2020 and 2019, are as follows:

Lease Cost	Classification	Three Months Ended September 30,		Nine Months Ended September 30,	
		2020	2019	2020	2019
		(in thousands)		(in thousands)	
Operating lease cost	Selling, general and administrative expenses	\$ 1,593	\$ 1,456	\$ 5,062	\$ 4,603
Short-term lease cost	Selling, general and administrative expenses	463	392	2,426	1,746
Variable lease cost	Selling, general and administrative expenses	262	230	1,119	626
Sublease income	Other income, net	(11)	(182)	(125)	(546)
Net lease cost		<u>\$ 2,307</u>	<u>\$ 1,896</u>	<u>\$ 8,482</u>	<u>\$ 6,429</u>

The weighted-average remaining term of the Company's operating leases was 2.1 years and the weighted-average discount rate used to measure the present value of the Company's operating lease liabilities was 3.9% as of September 30, 2020.

Cash payments included in the measurement of the Company's operating lease liabilities reported in operating cash flows were \$5.8 million and \$5.5 million for the nine months ended September 30, 2020 and 2019, respectively. During the nine months ended September 30, 2020, the Company obtained ROU assets of \$1.2 million in exchange for new operating lease obligations of \$1.2 million.

[Table of Contents](#)

Maturities of the Company's operating lease liabilities, which do not include short-term leases, as of September 30, 2020 are as follows:

<b>Maturity of Lease Liabilities</b>	<b>Operating leases</b>
	<b>(in thousands)</b>
2020	\$ 1,788
2021	6,676
2022	2,451
2023	940
2024	392
Thereafter	—
Total lease payments	12,247
Less: Present value discount	(815)
Total operating lease liabilities	\$ 11,432

## 9. Accounts Payable, Accrued Expenses and Other Liabilities

Accounts payable, accrued expenses and other liabilities consisted of the following:

	<b>September 30, 2020</b>	<b>December 31, 2019</b>
	<b>(in thousands)</b>	
Accounts payable	\$ 15,468	\$ 18,975
Accrued employee compensation	25,991	26,483
Accrued contracted services	64,748	74,486
Accrued restructuring	11,962	—
Accrued rebates, discounts and other incentives	27,286	21,529
Operating lease liabilities	6,133	6,456
Other liabilities	6,086	6,039
Accounts payable, accrued expenses and other liabilities	\$ 157,674	\$ 153,968

### **Research & Development Tax Credit**

The Company has benefited from the U.K. Small and Medium-sized Enterprise R&D Tax Credit scheme, or the SME scheme, under which it can obtain a tax credit of up to 33.4% of eligible research and development expenses incurred by the Company in the U.K.. Eligible expenses generally include employment costs for research staff, consumables, software and certain internal overhead costs incurred as part of research projects.

The Company has submitted claims seeking to obtain tax credits for qualifying R&D expenses incurred in the 2015, 2016 and 2017 calendar years. As described further in Note 13, the 2015 and 2016 claim was finalized during the quarter ended June 30, 2020, and therefore the \$10.5 million payment received in September 2019, which was previously deferred, was released into income.

With respect to the 2017 claim, in June 2020, the Company received a payment of \$9.4 million from Her Majesty's Revenue and Customs ("HMRC"), the U.K.'s government tax authority. Given the claim review has not been finalized for the 2017 year, the \$9.4 million credit received along with an additional \$0.4 million due to foreign currency translation are recorded as a deferred liability within Accounts payable, accrued expenses, and other liabilities.

## 10. Long-Term Debt

Debt, net of discounts and deferred financing costs, consisted of the following:

	<u>September 30, 2020</u>	<u>December 31, 2019</u>
	(in thousands)	
2023 Convertible Notes	\$ 460,000	\$ 460,000
2026 Convertible Notes	230,000	230,000
Long-term debt, gross	690,000	690,000
Less: Unamortized debt discounts and fees	(136,784)	(157,922)
Long-term debt, net	<u>\$ 553,216</u>	<u>\$ 532,078</u>

### 2019 Offering

On May 14, 2019, the Company issued and sold \$230.0 million aggregate principal amount of 2.00% Convertible Senior Notes due 2026 (the “2026 Convertible Notes”). The Company received net proceeds from the sale of the 2026 Convertible Notes of \$223.4 million, after deducting underwriting discounts, commissions and estimated offering expenses of approximately \$6.6 million.

The 2026 Convertible Notes were issued pursuant to a Second Supplemental Indenture, dated as of May 14, 2019 (the “Second Supplemental Indenture”), which supplements the Indenture (the “Base Indenture”), as supplemented by a First Supplemental Indenture (the “First Supplemental Indenture” and collectively with the Base Indenture and the Second Supplemental Indenture, the “Indenture”), each dated as of July 6, 2016, by and between the Company and U.S. Bank National Association, as trustee. The 2026 Convertible Notes are senior unsecured obligations of the Company, bear interest at a fixed rate of 2.00% per annum (payable semi-annually on May 15 and November 15 of each year, beginning on November 15, 2019) and will mature on May 15, 2026, unless earlier repurchased, redeemed or converted. Holders may convert their 2026 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding February 15, 2026 only under the following circumstances: (i) during any calendar quarter (and only during such calendar quarter) commencing after the calendar quarter ended on June 30, 2019, if the last reported sale price of the Company’s common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day; (ii) during the five business day period after any five consecutive trading day period in which the trading price (as defined in the Indenture) per \$1,000 principal amount of 2026 Convertible Notes for each trading day of such five consecutive trading day period was less than 98% of the product of the last reported sale price of the Company’s common stock and the conversion rate on each such trading day; (iii) if the Company calls any or all of the 2026 Convertible Notes for redemption, at any time prior to the close of business on the scheduled trading day immediately preceding the redemption date; or (iv) upon the occurrence of specified corporate events. On or after February 15, 2026 until the close of business on the business day immediately preceding the maturity date, holders may convert their 2026 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion of the 2026 Convertible Notes, the Company will pay or deliver, as the case may be, cash, shares of the Company’s common stock (and cash in lieu of any fractional shares) or a combination of cash and shares of the Company’s common stock, at the Company’s election. The initial conversion rate of the 2026 Convertible Notes is 9.2123 shares of the Company’s common stock per \$1,000 principal amount of 2026 Convertible Notes, which is equivalent to an initial conversion price of approximately \$108.55 per share of the Company’s common stock. The conversion rate is subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest. In addition, following certain corporate events that occur prior to the maturity date, the Company will increase the conversion rate for a holder who elects to convert its 2026 Convertible Notes in connection with such a corporate event in certain circumstances. The Company may not redeem the 2026 Convertible Notes prior to May 20, 2023. The Company may redeem for cash all or any portion of the 2026 Convertible Notes, at the Company’s option, on or after May 20, 2023, if the last reported sale price of the Company’s common stock has been at least 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption at a redemption price equal to 100% of the principal amount of the 2026 Convertible Notes to be redeemed,

plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the 2026 Convertible Notes. If the Company undergoes a fundamental change (as defined in the Indenture), holders may require the Company to repurchase for cash all or any portion of their 2026 Convertible Notes at a fundamental change repurchase price equal to 100% of the principal amount of the 2026 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date. The Indenture provides for customary events of default.

In accordance with Accounting Standards Codification (“ASC”) Subtopic 470-20, “Debt with Conversion and Other Options” (“ASC 470-20”), the Company used an effective interest rate of 9.9% to determine the liability component of the 2026 Convertible Notes. This resulted in the recognition of \$137.5 million as the liability component of the 2026 Convertible Notes and the recognition of the residual \$85.9 million as the debt discount with a corresponding increase to additional paid-in capital for the equity component of the 2026 Convertible Notes. The underwriting discount and estimated offering expenses totaling \$6.6 million were allocated between the debt and equity issuance costs in proportion to the allocation of the liability and equity components of the 2026 Convertible Notes. Accordingly, equity issuance costs of \$2.5 million were recorded as an offset to additional paid-in capital and total debt issuance costs of \$4.1 million were recorded on the issuance date and are reflected in the unaudited condensed consolidated balance sheet as a direct deduction from the carrying value of the associated debt liability. The debt discount and debt issuance costs will be amortized as non-cash interest expense through May 15, 2026.

The fair value of the 2026 Convertible Notes was approximately \$159.4 million and \$294.9 million at September 30, 2020 and December 31, 2019, respectively, and was determined using Level 2 inputs based on quoted market values.

### **2016 Offerings**

On July 6, 2016, the Company issued and sold \$460.0 million aggregate principal amount of 3.25% Convertible Senior Notes due 2023 (the “2023 Convertible Notes”, and together with the 2026 Convertible Notes, the “Convertible Notes”). The Company received net proceeds from the sale of the 2023 Convertible Notes of \$447.6 million, after deducting underwriting discounts, commissions and estimated offering expenses of approximately \$12.4 million. The Company used approximately \$38.4 million of such net proceeds to fund the cost of the Capped Call Transactions (as defined below) that were entered into in connection with the issuance of the 2023 Convertible Notes.

The 2023 Convertible Notes were issued pursuant to the Base Indenture, as supplemented by the First Supplemental Indenture. The 2023 Convertible Notes are senior unsecured obligations of the Company, bear interest at a fixed rate of 3.25% per year (payable semi-annually on January 1 and July 1 of each year, beginning on January 1, 2017) and will mature on July 1, 2023, unless earlier repurchased, redeemed or converted. Holders may convert their 2023 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding January 1, 2023 only under the following circumstances: (i) during any calendar quarter commencing after the calendar quarter ended on September 30, 2016, if the last reported sale price of the Company’s common stock for at least 20 trading days (whether or not consecutive) during the period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day; (ii) during the five business day period after any five consecutive trading day period in which the trading price (as defined in the Indenture) per \$1,000 principal amount of 2023 Convertible Notes for each trading day of such five consecutive trading day period was less than 98% of the product of the last reported sale price of the Company’s common stock and the conversion rate on each such trading day; (iii) if the Company calls any or all of the 2023 Convertible Notes for redemption, at any time prior to the close of business on the scheduled trading day immediately preceding the redemption date; or (iv) upon the occurrence of specified corporate events. On or after January 1, 2023 until the close of business on the second scheduled trading day immediately preceding the maturity date, holders may convert their 2023 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion of the 2023 Convertible Notes, the Company will pay or deliver, as the case may be, cash, shares of the Company’s common stock (and cash in lieu of any fractional shares) or a combination of cash and shares of the Company’s common stock, at the Company’s election. The initial conversion rate of the 2023 Convertible Notes is 5.0358 shares of the Company’s common stock per \$1,000 principal amount of 2023 Convertible Notes, which is equivalent to an initial conversion price of approximately \$198.58 per share of the Company’s common stock. The conversion rate is subject to adjustment upon the occurrence of certain events but will not be adjusted for any accrued and unpaid interest. If the Company undergoes a fundamental change (as defined in the Indenture), holders may require the Company to repurchase for cash all or any portion of their 2023

Convertible Notes at a fundamental change repurchase price equal to 100% of the principal amount of the 2023 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date. In addition, if certain make-whole fundamental changes occur, the Company will, in certain circumstances, increase the conversion rate for any 2023 Convertible Notes converted in connection with such make-whole fundamental change. The Company may not redeem the 2023 Convertible Notes prior to July 6, 2021. The Company may redeem for cash all or part of the 2023 Convertible Notes, at its option, on or after July 6, 2021, if the last reported sale price of the Company's common stock has been at least 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption at a redemption price equal to 100% of the principal amount of the 2023 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. The Indenture provides for customary events of default.

On June 30, 2016, in connection with the pricing of the 2023 Convertible Notes, the Company entered into privately-negotiated capped call transactions (the "Base Capped Call Transactions") with each of Royal Bank of Canada, UBS AG, London Branch, and Credit Suisse Capital LLC (the "Option Counterparties"). On July 1, 2016, in connection with the underwriters' exercise of their over-allotment option in full, the Company entered into additional capped call transactions (the "Additional Capped Call Transactions" and, together with the Base Capped Call Transactions, the "Capped Call Transactions") with the Option Counterparties. The Capped Call Transactions are expected generally to reduce the potential dilution with respect to the Company's common stock and/or offset the cash payments the Company would be required to make in excess of the principal amount of converted 2023 Convertible Notes, as the case may be, upon conversion of the 2023 Convertible Notes in the event that the market price per share of the Company's common stock, as measured under the terms of the Capped Call Transactions, is greater than the strike price of the Capped Call Transactions, which initially corresponds to the conversion price of the 2023 Convertible Notes and is subject to anti-dilution adjustments substantially similar to those applicable to the conversion rate of the 2023 Convertible Notes. The cap price of the Capped Call Transactions is initially \$262.2725 per share, and is subject to certain adjustments under the terms of the Capped Call Transactions. If, however, the market price per share of the Company's common stock, as measured under the terms of the Capped Call Transactions, exceeds the cap price of the Capped Call Transactions, there would nevertheless be dilution and/or there would not be an offset of such potential cash payments, in each case, upon conversion of the Convertible Notes to the extent that such market price exceeds the cap price of the Capped Call Transactions.

In accordance with ASC 470-20, the Company used an effective interest rate of 8.4% to determine the liability component of the 2023 Convertible Notes. This resulted in the recognition of \$334.4 million as the liability component of the 2023 Convertible Notes and the recognition of the residual \$113.1 million as the debt discount with a corresponding increase to additional paid-in capital for the equity component of the 2023 Convertible Notes.

The fair value of the 2023 Convertible Notes was approximately \$370.3 million and \$463.5 million at September 30, 2020 and December 31, 2019, respectively, and was determined using Level 2 inputs based on quoted market values.

#### ***Interest Expense on Convertible Notes***

Interest expense was \$12.1 million and \$11.8 million for the three months ended September 30, 2020 and 2019, respectively, and \$35.8 million and \$29.5 million for the nine months ended September 30, 2020 and 2019, respectively, related to the Convertible Notes. Accrued interest on the Convertible Notes was approximately \$5.5 million and \$8.1 million as of September 30, 2020 and December 31, 2019, respectively. The Company recorded debt issuance costs of \$19.0 million, which are being amortized using the effective interest method. As of September 30, 2020, \$11.3 million of debt issuance costs are recorded on the unaudited condensed consolidated balance sheet in Long-term debt. Cash payments for interest were \$17.3 million and \$7.5 million for the nine months ended September 30, 2020 and 2019, respectively.

#### **11. Product Revenue, Net**

The Company recognized net sales of Ocaliva of \$79.5 million and \$61.5 million for the three months ended September 30, 2020 and 2019, respectively and \$229.4 million and \$179.3 million for the nine months ended September 30, 2020 and 2019, respectively.

The table below summarizes consolidated product revenue, net by region:

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
	<u>(in thousands)</u>		<u>(in thousands)</u>	
Product revenue, net:				
U.S.	\$ 58,643	\$ 45,232	\$ 169,051	\$ 133,914
ex-U.S.	20,878	16,313	60,371	45,372
Total product revenue, net	<u>\$ 79,521</u>	<u>\$ 61,545</u>	<u>\$ 229,422</u>	<u>\$ 179,286</u>

## 12. License Agreement

### *Sumitomo Dainippon Pharma Co., Ltd.*

In March 2011, the Company entered into an exclusive license agreement (the “Original Sumitomo Agreement”) with Sumitomo Dainippon Pharma Co., Ltd. (“Sumitomo Dainippon”), pursuant to which the Company granted to Sumitomo Dainippon an exclusive license to research, develop and commercialize OCA for the treatment of PBC and NASH in Japan and China (excluding Taiwan) and an option to research, develop and commercialize OCA in certain countries outside of such territories (the “Country Option”). The Company received an upfront payment from Sumitomo Dainippon of \$15.0 million under the terms of the Original Sumitomo Agreement. In October 2019, the Company and Sumitomo Dainippon mutually agreed to terminate with immediate effect the Sumitomo Agreement. In connection with the termination of the Sumitomo Agreement, Sumitomo Dainippon agreed to return to the Company the rights to develop and commercialize OCA in China and the Company agreed to forego any further milestone or royalty payments relating to the development and commercialization of OCA in China. No payment is due from the Company to Sumitomo Dainippon as a result of the termination of the Sumitomo Agreement.

The Company recognized licensing revenue of \$0 and \$0.4 million for the three months ended September 30, 2020 and 2019, respectively, and \$0 and \$1.2 million for the nine months ended September 30, 2020 and 2019, respectively, under the Sumitomo Agreement.

## 13. Research and Development Tax Credit

The Company has benefited from the U.K. Small and Medium-sized Enterprise R&D Tax Credit scheme, or the SME scheme, under which it can obtain a tax credit of up to 33.4% of eligible research and development expenses incurred by the Company in the U.K.. Eligible expenses generally include employment costs for research staff, consumables, software and certain internal overhead costs incurred as part of research projects.

The Company submitted a claim seeking to obtain tax credits for qualifying R&D expenses incurred in the years ended December 31, 2015 and 2016. In September 2019, the Company received a partial payment of \$10.5 million from HMRC. In April 2020, the Company received the remaining payment for the 2015 and 2016 claim years of \$11.3 million.

The claim for 2015 and 2016 was finalized and approved in the quarter ended June 30, 2020, at which time the Company recorded the U.K. research and development tax credit payments received of \$22.0 million as a reduction of research and development expense in the condensed consolidated statements of operations.

## 14. Restructuring Expenses

On August 31, 2020, the Company adopted a plan to reduce its workforce in light of the previously announced receipt of a complete response letter from the U.S. Food and Drug Administration (the “FDA”) with respect to its New Drug Application for OCA for the treatment of liver fibrosis due to NASH (the “2020 Workforce Plan”). The 2020 Workforce Plan seeks to streamline the Company’s operations and reduce operating expenses, while maintaining the critical resources needed to continue to support the NASH and PBC clinical programs, pursue the approval of OCA for the treatment of liver fibrosis due to NASH and support the Company’s successful PBC business. The 2020 Workforce Plan will result in a workforce reduction of approximately 25%, or approximately 170 employees. The 2020 Workforce

[Table of Contents](#)

Plan was implemented during the third quarter of 2020, immediately after its announcement, and is expected to be substantially completed by the end of 2020 upon payments of cash for charges incurred under the 2020 Workforce Plan. In the three and nine months ended September 30, 2020 the Company recorded restructuring charges of \$13.4 million, which were primarily related to severance costs and other related termination benefits incurred in conjunction with the 2020 Workforce Plan.

The following table reflects total expenses related to restructuring activities recognized within the Condensed Consolidated Statements of Operations as restructuring costs:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020		2020	
	(in thousands)			
Employee compensation costs	\$	11,973	\$	11,973
Equity compensation costs		1,408		1,408
Total restructuring costs	\$	13,381	\$	13,381

The Company recorded \$1.4 million in non-cash stock-based compensation expense in association with the acceleration of the vesting of certain options and RSUs held by terminated employees.

The following table displays a rollforward of the changes to the accrued balances as of September 30, 2020:

	Severance and Related Costs	
	(in thousands)	
Accrued balance at December 31, 2019	\$	—
Charges incurred		11,962
Cash payments made		—
Other reserve adjustments		—
Accrued balance at September 30, 2020	\$	11,962

## 15. Stockholders' Equity

### *Increase in Authorized Shares of Common Stock*

On May 28, 2020, at the 2020 Annual Meeting of Stockholders, the Company obtained approval from its stockholders to increase the number of authorized shares of the Company's common stock, par value \$0.001, from 45,000,000 shares to 90,000,000 shares. The increase in the authorized shares of common stock was effected pursuant to a Certificate of Amendment to the Company's restated certificate of incorporation, filed with the Secretary of the State of Delaware on May 28, 2020.

### *2019 Public Offering and Concurrent Private Placement*

On May 14, 2019, the Company issued and sold (i) 2,760,000 shares of common stock in a registered public offering (including 360,000 shares issued and sold upon the exercise in full of the underwriters' option to purchase additional shares), at a price to the public of \$83.50 per share (the "2019 Public Offering") and (ii) 119,760 shares of common stock (the "2019 Private Placement Shares") in a concurrent private placement of common stock (the "2019 Concurrent Private Placement") exempt from the registration requirements of the Securities Act of 1933, as amended (the "Securities Act"), at a purchase price per share equivalent to the price to the public set in the 2019 Public Offering and pursuant to a securities purchase agreement (the "2019 Securities Purchase Agreement") that the Company entered into with Samsara BioCapital, L.P. ("Samsara"), one of the Company's existing stockholders. Pursuant to the 2019 Securities Purchase Agreement, the Company granted to Samsara certain registration rights requiring the Company, upon request of Samsara on or after July 9, 2019 and subject to certain terms and conditions, to register the resale by Samsara of its 2019 Private Placement Shares. Such registration rights have since expired.

## 16. Stock Compensation

The Company's 2012 Equity Incentive Plan ("2012 Plan") became effective upon the pricing of its initial public offering in October 2012. At the same time, the Company's 2003 Stock Incentive Plan ("2003 Plan") was terminated and 555,843 shares available under the 2003 Plan were added to the 2012 Plan.

On January 1, 2020, the number of shares available for issuance under the 2012 Plan increased by 1,211,533 shares, as a result of the automatic increase provisions thereof.

The estimated fair value of the stock options granted in the nine months ended September 30, 2020 was determined utilizing a Black-Scholes option-pricing model at the date of grant. The fair value of the restricted stock units ("RSUs") granted in the nine months ended September 30, 2020 was determined utilizing the closing price of the Company's common stock on the date of grant. The fair value of the performance restricted stock units ("PRSUs") granted in the nine months ended September 30, 2020 was determined utilizing the Monte Carlo simulation method.

The following table summarizes stock option activity during the nine months ended September 30, 2020:

	Number of Options (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2019	1,981	\$ 99.87	7.4	\$ 65,662
Granted	688	\$ 93.13	—	\$ —
Exercised	(22)	\$ 52.20	—	\$ —
Cancelled/forfeited	(139)	\$ 95.70	—	\$ —
Expired	(73)	\$ 145.34	—	\$ —
Outstanding at September 30, 2020	2,435	\$ 97.27	7.3	\$ 1,502
Expected to vest	1,144	\$ 89.99	8.7	\$ —
Exercisable	1,291	\$ 103.72	6.1	\$ 1,502

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the underlying options and the deemed fair value of the Company's common stock for those options that had exercise prices lower than the deemed fair value of the Company's common stock. As of September 30, 2020, the total compensation cost related to non-vested option awards not yet recognized is approximately \$52.9 million with a weighted average remaining vesting period of 1.27 years.

The Company estimated the fair value of stock options granted in the periods presented utilizing a Black-Scholes option-pricing model utilizing the following assumptions:

	Nine Months Ended September 30,	
	2020	2019
Volatility	61.9 - 87.1 %	87.0 - 89.9 %
Expected term (in years)	5.5 - 6.0	5.5 - 6.0
Risk-free rate	0.2 - 1.7 %	1.4 - 2.9 %
Expected dividend yield	— %	— %

[Table of Contents](#)

The following table summarizes the aggregate RSU, restricted stock award (“RSA”), PRSU and performance restricted share award (“PRSA”) activity during the nine months ended September 30, 2020:

	Number of Awards (in thousands)	Weighted Average Grant Date Fair Value
Non-vested awards at December 31, 2019	709	\$ 88.39
Granted	698	\$ 80.18
Vested	(186)	\$ 77.93
Forfeited	(92)	\$ 93.82
Non-vested awards at September 30, 2020	<u>1,129</u>	<u>\$ 84.27</u>

As of September 30, 2020, there is approximately \$70.4 million of total unrecognized compensation expense related to unvested RSUs, RSAs, PRSUs and PRSAs, which is expected to be recognized over a weighted average vesting period of 1.41 years.

During the nine months ended September 30, 2020, the Company granted a total of 64,900 PRSUs to certain of the Company’s executive officers. The performance criterion for such PRSUs is based on the Total Shareholder Return (“TSR”) of the Company’s common stock relative to the TSR of the companies comprising the S&P Biotechnology Select Industry Index (the “TSR Peer Group”) over a 3-year performance period and is accounted for as a market condition under ASC Topic 718, *Compensation – Stock Compensation*. The TSR for the Company or a member of the TSR Peer Group is calculated by dividing (a) the difference of the ending average stock price minus the beginning average stock price by (b) the beginning average stock price. The beginning average stock price equals the average closing stock price over the one calendar month period prior to the beginning of the performance period, after adjusting for dividends, as applicable. The ending average stock price equals the average closing price over the one calendar month period ending on the last day of the performance period, after adjusting for dividends, as applicable. The Company’s relative TSR is then used to calculate the payout percentage, which may range from zero percent (0%) to one hundred and fifty percent (150%) of the target award. The Company utilized a Monte Carlo Simulation to determine the grant date fair value of such PRSUs.

The Company recorded approximately \$1.9 million of stock-based compensation related to such PRSUs during the nine months ended September 30, 2020.

The Company accounts for all forfeitures when they occur. Ultimately, the actual expense recognized over the vesting period will be for only those shares that vest and are not forfeited. The Company has in the past, and may in the future, grant performance-based awards with vesting terms based on the achievement of specified goals. To the extent such awards do not contain a market condition, the Company recognizes no expense until achievement of the performance requirement is deemed probable. There are no awards with performance conditions outstanding as of September 30, 2020.

Stock-based compensation expense has been reported in the Company’s condensed consolidated statements of operations as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
Selling, general and administrative	\$ 11,119	\$ 10,104	\$ 33,091	\$ 33,029
Research and development	3,298	3,026	9,882	9,780
Restructuring	1,408	—	1,408	—
Total stock-based compensation	<u>\$ 15,825</u>	<u>\$ 13,130</u>	<u>\$ 44,381</u>	<u>\$ 42,809</u>

## 17. Net Loss Per Share

Basic loss per share is computed by dividing net loss attributable to common stockholders (numerator) by the weighted average number of common shares outstanding (denominator) during the period. For the three and nine-month periods

ended September 30, 2020 and 2019, as the Company was in a net loss position, the diluted loss per share computations for such periods did not assume the conversion of the Convertible Notes, exercise of stock options or vesting of RSUs or PRSUs as they would have had an anti-dilutive effect on loss per share.

The following potentially dilutive securities have been excluded from the computations of diluted weighted average shares outstanding for the three and nine-month periods ended September 30, 2020 and 2019, as the inclusion thereof would have been anti-dilutive:

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
	<u>(in thousands)</u>		<u>(in thousands)</u>	
Shares issuable upon conversion of Convertible Notes	4,435	4,435	4,435	4,435
Options	2,464	2,086	2,436	2,086
Unvested restricted stock units	940	597	880	597
Total	<u>7,839</u>	<u>7,118</u>	<u>7,751</u>	<u>7,118</u>

## 18. Commitments and Contingencies

### Legal Proceedings

The Company is involved in various disputes, legal proceedings and litigation in the course of its business, including the matters described below and, from time to time, governmental inquiries and investigations and employment and other litigation. These matters, which could result in damages, fines or other administrative, civil or criminal remedies, liabilities or penalties, are often complex and the outcome of such matters is often uncertain. The Company may from time to time enter into settlements to resolve such matters.

#### Shareholder Litigation

On September 27, 2017, a purported shareholder class action, initially styled *DeSmet v. Intercept Pharmaceuticals, Inc., et al.*, was filed in the United States District Court for the Southern District of New York, naming the Company and certain of its officers as defendants. The Court appointed lead plaintiffs in the lawsuit on June 1, 2018, and the lead plaintiffs filed an amended complaint on July 31, 2018, captioned *Hou Liu and Amy Fu v. Intercept Pharmaceuticals, Inc., et al.*, naming the Company and certain of its current and former officers as defendants. The lead plaintiffs claim to be suing on behalf of anyone who purchased or otherwise acquired the Company's common stock between June 9, 2016 and September 20, 2017. This lawsuit alleges that material misrepresentations and/or omissions of material fact were made in the Company's public disclosures during the period from June 9, 2016 to September 20, 2017, in violation of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Rule 10b-5 promulgated thereunder. The alleged improper disclosures relate to statements regarding Ocaliva dosing, use and pharmacovigilance-related matters, as well as the Company's operations, financial performance and prospects. The plaintiffs seek unspecified monetary damages on behalf of the putative class, an award of costs and expenses, including attorney's fees, and rescissory damages. On September 14, 2018, the Company filed a motion to dismiss the amended complaint. On March 26, 2020, the Court granted the Company's motion to dismiss the amended complaint in its entirety, and on March 27, 2020 the Court entered judgment in favor of the Company. On May 8, 2020, the plaintiffs filed a motion to set aside the judgment and grant leave to file a second amended complaint. On September 9, 2020, the Court denied the plaintiffs' motion to set aside the judgment and grant leave to file a second amended complaint, finding that the proposed second amended complaint did not cure the deficiencies identified in the amended complaint. On October 9, 2020, the plaintiffs filed a notice of appeal to the United States Court of Appeals for the Second Circuit, challenging the March 27, 2020 judgment and several orders entered in this action.

Separately, on December 1, 2017, a purported shareholder demand was made on the Company based on substantially the same allegations as those set forth in the securities case above. In addition, on January 5, 2018, a follow-on derivative suit, styled *Davis v. Pruzanski, et al.*, was filed in New York state court by shareholder Gregg Davis based on substantially the same allegations as those set forth in the securities case above.

On November 5, 2020, a purported shareholder class action, styled *Chauhan v. Intercept Pharmaceuticals, Inc., et al.*, was filed in the United States District Court for the Eastern District of New York, naming the Company and certain of its officers as defendants. The plaintiff claims to be suing on behalf of anyone who purchased or otherwise acquired the Company's securities between September 28, 2019 and October 7, 2020. This lawsuit alleges that material misrepresentations and/or omissions of material fact were made in the Company's public disclosures during the period from September 28, 2019 to October 7, 2020, in violation of Sections 10(b) and 20(a) of the Exchange Act, and Rule 10b-5 promulgated thereunder. The alleged improper disclosures relate to statements regarding the Company's New Drug Application for OCA for the treatment of liver fibrosis due to NASH and the use of Ocaliva in patients with PBC, as well as the Company's operations, financial performance and prospects. The plaintiff seeks unspecified monetary damages on behalf of the putative class, and an award of costs and expenses, including attorney's fees.

While the Company believes that it has a number of valid defenses to the claims described above and intends to vigorously defend itself, the matters are in the early stages of litigation and no assessment can be made as to the likely outcome of the matters or whether they will be material to the Company. Accordingly, an estimate of the potential loss, or range of loss, if any, to the Company relating to the matters is not possible at this time.

### ***Patent Litigation***

The Company has received paragraph IV certification notice letters from five generic drug manufacturers indicating that each such manufacturer has submitted to the FDA an Abbreviated New Drug Application ("ANDA") seeking approval to manufacture and sell a generic version of the Company's 5 mg and 10 mg dosage strengths of Ocaliva® (obeticholic acid) for PBC prior to the expiration of certain patents protecting Ocaliva.

#### *Apotex Abbreviated New Drug Application*

In July 2020, the Company received a paragraph IV certification notice (the "Apotex PIV Notice") from Apotex Inc. ("Apotex") indicating that Apotex has submitted to the FDA an ANDA seeking approval to manufacture and sell a generic version of the Company's 5 mg and 10 mg dosage strengths of Ocaliva® (obeticholic acid) for PBC prior to the expiration of the Company's U.S. Patents Nos. 9,238,673 (the "'673 Patent"), 10,047,117 (the "'117 Patent"), 10,052,337 (the "'337 Patent"), and 10,174,073 (the "'073 Patent"), and collectively with the '673 Patent, '117 Patent and '337 Patent, the "Apotex Challenged Patents"), which are listed for Ocaliva in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (referred to as the "Orange Book"). The Apotex PIV Notice alleges that the Apotex Challenged Patents are invalid, unenforceable, and/or will not be infringed by the commercial manufacture, use or sale of the generic products described in Apotex's ANDA. Apotex did not make a paragraph IV certification against the Company's U.S. Patents Nos. 7,138,390 (the "'390 Patent"), 8,058,267 (the "'267 Patent") or 8,377,916 (the "'916 Patent"), which are also listed for Ocaliva in the Orange Book. The Company initiated a patent infringement suit against Apotex in the United States District Court for the District of Delaware within 45 days of receipt of the Apotex PIV Notice. As a result, under the Drug Price Competition and Patent Term Restoration Act of 1984 (the "Hatch-Waxman Act"), the FDA cannot grant final approval to Apotex's ANDA before November 27, 2023 or a court decision in Apotex's favor, whichever is earlier. Recently, the U.S. Patent and Trademark Office (the "USPTO") awarded the Company two additional Orange Book-listable patents that protect Ocaliva: U.S. Patents Nos. 10,751,349 (the "'349 Patent") and 10,758,549 (the "'549 Patent"). In September and October 2020, the Company received amended paragraph IV certification notices from Apotex challenging the '349 Patent and the '549 Patent, respectively, that contain allegations similar to those in the Apotex PIV Notice. The Company is evaluating its legal options, including asserting the '349 Patent and the '549 Patent against Apotex.

#### *Lupin Abbreviated New Drug Application*

In July 2020, the Company received a paragraph IV certification notice (the "Lupin PIV Notice") from Lupin Limited ("Lupin") indicating that Lupin has submitted to the FDA an ANDA seeking approval to manufacture and sell a generic version of the Company's 5 mg and 10 mg dosage strengths of Ocaliva® (obeticholic acid) for PBC prior to the expiration of the '390 Patent, the '673 Patent, the '117 Patent, the '337 Patent and the '073 Patent (collectively, the "Lupin Challenged Patents"), which are listed for Ocaliva in the FDA's Orange Book. The Lupin PIV Notice alleges that the Lupin Challenged Patents are invalid, unenforceable, and/or will not be infringed by the commercial manufacture, use or sale of the generic products described in Lupin's ANDA. Lupin did not make a paragraph IV certification against the '267 Patent or the '916

Patent, which are also listed for Ocaliva in the Orange Book. The Company initiated a patent infringement suit against Lupin in the United States District Court for the District of Delaware within 45 days of receipt of the Lupin PIV Notice. As a result, under the Hatch-Waxman Act, the FDA cannot grant final approval to Lupin's ANDA before November 27, 2023 or a court decision in Lupin's favor, whichever is earlier. In September 2020, the Company received an amended paragraph IV certification notice from Lupin challenging the '349 Patent and the '549 Patent that contains allegations similar to those in the Lupin PIV Notice. The Company is evaluating its legal options, including asserting the '349 Patent and the '549 Patent against Lupin.

*Amneal Abbreviated New Drug Application*

In July 2020, the Company received a paragraph IV certification notice (the "Amneal PIV Notice") from Amneal Pharmaceuticals of New York, LLC, as U.S. agent for Amneal EU Limited ("Amneal"), indicating that Amneal has submitted to the FDA an ANDA seeking approval to manufacture and sell a generic version of the Company's 5 mg and 10 mg dosage strengths of Ocaliva® (obeticholic acid) for PBC prior to the expiration of the '673 Patent, the '117 Patent, the '337 Patent and the '073 Patent (collectively, the "Amneal Challenged Patents"), which are listed for Ocaliva in the FDA's Orange Book. The Amneal PIV Notice alleges that the Amneal Challenged Patents are invalid, unenforceable, and/or will not be infringed by the commercial manufacture, use or sale of the generic products described in Amneal's ANDA. Amneal did not make a paragraph IV certification against the '390 Patent, the '267 Patent or the '916 Patent, which are also listed for Ocaliva in the Orange Book. The Company initiated a patent infringement suit against Amneal in the United States District Court for the District of Delaware within 45 days of receipt of the Amneal PIV Notice. As a result, under the Hatch-Waxman Act, the FDA cannot grant final approval to Amneal's ANDA before November 27, 2023 or a court decision in Amneal's favor, whichever is earlier. In October 2020, the Company received an amended paragraph IV certification notice from Amneal challenging the '349 Patent and the '549 Patent that contains allegations similar to those in the Amneal PIV Notice. The Company is evaluating its legal options, including asserting the '349 Patent and the '549 Patent against Amneal.

*Optimus Abbreviated New Drug Application*

In July 2020, the Company received a paragraph IV certification notice (the "Optimus PIV Notice") from Optimus Pharma Pvt Ltd ("Optimus") indicating that Optimus has submitted to the FDA an ANDA seeking approval to manufacture and sell a generic version of the Company's 5 mg and 10 mg dosage strengths of Ocaliva® (obeticholic acid) for PBC prior to the expiration of the '390 Patent, the '673 Patent, the '117 Patent, the '337 Patent and the '073 Patent (collectively, the "Optimus Challenged Patents") which are listed for Ocaliva in the FDA's Orange Book. The Optimus PIV Notice alleges that the Optimus Challenged Patents are invalid, unenforceable, and/or will not be infringed by the commercial manufacture, use or sale of the generic products described in Optimus's ANDA. Optimus did not make a paragraph IV certification against the '267 Patent or the '916 Patent, which are also listed for Ocaliva in the Orange Book. The Company initiated a patent infringement suit against Optimus in the United States District Court for the District of Delaware within 45 days of receipt of the Optimus PIV Notice. As a result, under the Hatch-Waxman Act, the FDA cannot grant final approval to Optimus's ANDA before November 27, 2023 or a court decision in Optimus's favor, whichever is earlier. In October 2020, the Company received an amended paragraph IV certification notice from Optimus challenging the '349 Patent and the '549 Patent that contains allegations similar to those in the Optimus PIV Notice. The Company is evaluating its legal options, including asserting the '349 Patent and the '549 Patent against Optimus.

*MSN Abbreviated New Drug Application*

In July 2020, the Company received a paragraph IV certification notice (the "MSN PIV Notice") from MSN Pharmaceuticals Inc. and MSN Laboratories Private Limited (collectively, "MSN") indicating that MSN has submitted to the FDA an ANDA seeking approval to manufacture and sell a generic version of the Company's 5 mg and 10 mg dosage strengths of Ocaliva® (obeticholic acid) for PBC prior to the expiration of the '390 Patent, the '673 Patent, the '117 Patent, the '337 Patent and the '073 Patent (collectively, the "MSN Challenged Patents") which are listed for Ocaliva in the FDA's Orange Book. The MSN PIV Notice alleges that the MSN Challenged Patents are invalid, unenforceable, and/or will not be infringed by the commercial manufacture, use or sale of the generic products described in MSN's ANDA. MSN did not make a paragraph IV certification against the '267 Patent or the '916 Patent, which are also listed for Ocaliva in the Orange Book. The Company initiated a patent infringement suit against MSN in the United States District Court for the District

of Delaware within 45 days of receipt of the MSN PIV Notice. As a result, under the Hatch-Waxman Act, the FDA cannot grant final approval to MSN's ANDA before November 27, 2023 or a court decision in MSN's favor, whichever is earlier.

In October 2020, the USPTO granted to the Company a reissue patent, U.S. Patent No. RE 48,286 (the "'286 Patent"). By operation of law, the '390 Patent was withdrawn and replaced by the '286 Patent, which contains composition of matter claims to OCA and has the same term as the '390 Patent. In November 2020, the Company was informed by the USPTO that its petition for a five-year patent term extension of the '286 Patent had been granted and that the '286 Patent will now expire in 2027. The '286 Patent has been listed in the Orange Book, and will be substituted for the '390 Patent in the Lupin, Optimus and MSN litigation described above.

These proceedings are costly and time consuming. Successful challenges to the Company's patent or other intellectual property rights through these proceedings could result in a loss of rights in the relevant jurisdiction and may allow third parties to use the Company's proprietary technologies without a license from the Company or its collaborators. While the Company intends to vigorously defend and enforce its intellectual property rights protecting Ocaliva, the Company can offer no assurance as to when the lawsuits will be decided, whether the lawsuits will be successful, or that a generic equivalent of Ocaliva will not be approved and enter the market before the expiration of the Company's patents.

## Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations.

*You should read the following discussion and analysis together with our condensed consolidated financial statements and accompanying notes included elsewhere in this Quarterly Report on Form 10-Q and our audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2019 (the “Annual Report”). This discussion and analysis contains forward-looking statements, which involve risks and uncertainties. As a result of many factors, such as those described under “Cautionary Note Regarding Forward-Looking Statements,” “Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q and in our Annual Report, our actual results may differ materially from those anticipated in these forward-looking statements.*

### Overview

We are a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases with high unmet medical need utilizing our proprietary bile acid chemistry. Our first marketed product, Ocaliva® (obeticholic acid or “OCA”), is a farnesoid X receptor (“FXR”) agonist approved in the United States, the European Union and several other jurisdictions for the treatment of primary biliary cholangitis (“PBC”) in combination with ursodeoxycholic acid (“UDCA”) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA. In addition to commercializing OCA for PBC under the Ocaliva brand name, we are currently developing OCA for additional indications, including nonalcoholic steatohepatitis (“NASH”). We are also developing several other product candidates in various stages of clinical and preclinical development. We believe that OCA and our other product candidates have the potential to treat orphan and other more prevalent liver diseases such as NASH for which there are currently limited therapeutic options.

Ocaliva was approved for PBC by the U.S. Food and Drug Administration (“FDA”) in May 2016 under the accelerated approval pathway. We commenced sales and marketing of Ocaliva in the United States shortly after receiving approval, and Ocaliva is now available to U.S. patients primarily through a network of specialty pharmacy distributors. Ocaliva received conditional approval for PBC from the European Commission in December 2016 and we commenced our European commercial launch in January 2017. We have submitted dossiers and obtained, or are otherwise pursuing, reimbursement from a number of national authorities in Europe. Since January 2017, Ocaliva has also received regulatory approval in several of our target markets outside the United States and Europe, including Canada, Israel and Australia, and we are pursuing marketing approval of Ocaliva for PBC in our other international target markets. Ocaliva received orphan drug designation in both the United States and the European Union for the treatment of PBC.

Our lead product candidate is OCA for the potential treatment of NASH. In February 2019, we announced topline results from the planned 18-month interim analysis of our pivotal Phase 3 clinical trial of OCA in patients with liver fibrosis due to NASH, known as the REGENERATE trial. In the primary efficacy analysis, once-daily OCA 25 mg met the primary endpoint agreed with the FDA of fibrosis improvement by at least one stage with no worsening of NASH at the planned 18-month interim analysis. Adverse events were generally mild to moderate in severity and the most common were consistent with the known profile of OCA. Interim analysis results at 18 months were based on surrogate endpoints and the impact on clinical outcomes has not been confirmed. The REGENERATE trial is ongoing and is expected to continue through clinical outcomes for verification and description of the clinical benefit of OCA. OCA also achieved the primary endpoint in a Phase 2b clinical trial for the treatment of NASH that completed in late July 2014, known as the FLINT trial, which was sponsored by the U.S. National Institute of Diabetes and Digestive and Kidney Diseases, a part of the National Institutes of Health. OCA has received breakthrough therapy designation from the FDA for the treatment of NASH patients with liver fibrosis. In September 2019, we submitted a New Drug Application (“NDA”) to the FDA seeking accelerated approval of OCA for liver fibrosis due to NASH. In November 2019, the FDA accepted our NDA for filing and granted a priority review designation of OCA for liver fibrosis due to NASH. In December 2019, we submitted a Marketing Authorization Application (“MAA”) to the European Medicines Agency (the “EMA”) seeking conditional approval of OCA for liver fibrosis due to NASH. In January 2020, the EMA validated our MAA and thereby confirmed that our MAA was sufficiently complete to begin the formal review process. In June 2020, we received a complete response letter (“CRL”) from the FDA stating that our NDA for OCA for the treatment of liver fibrosis due to NASH could not be approved in its present form. The CRL indicated that, based on the data the FDA had reviewed, the FDA has determined that the predicted benefit of OCA based on a surrogate histopathologic endpoint remains uncertain and does not sufficiently

outweigh the potential risks to support accelerated approval for the treatment of patients with liver fibrosis due to NASH. At that time, the FDA recommended that we submit additional post-interim analysis efficacy and safety data from the ongoing REGENERATE trial in support of potential accelerated approval and that the long-term outcomes phase of the trial should continue.

As part of our product development activities, we expect to continue to invest in evaluating the potential of OCA in progressive non-viral liver diseases. We are currently conducting a Phase 3 clinical trial in NASH patients with compensated cirrhosis, known as the REVERSE trial. In January 2020, we announced that we completed enrollment of the REVERSE trial with over 900 patients randomized. We are also studying OCA in combination with bezafibrate, a pan-peroxisome proliferator-activated receptor agonist, in patients with PBC and potentially may study such combination in other liver diseases. In addition, we have other compounds in early stages of research and development in our pipeline.

## **Recent Developments**

### ***OCA for Liver Fibrosis due to NASH***

We are in discussions with the FDA with respect to the potential resubmission of our NDA seeking accelerated approval of OCA for the treatment of liver fibrosis due to NASH. We had our end of review meeting with the FDA in October 2020 to discuss the FDA's risk-benefit assessment in the CRL based on its review of the available data, as well as our proposed resubmission of our NDA for the treatment of liver fibrosis due to NASH. The meeting was constructive and the FDA has provided us with helpful guidance regarding supplemental data we can provide to further characterize OCA's efficacy and safety profile that could support resubmission based on our Phase 3 REGENERATE 18-month biopsy data, together with a safety update from our ongoing studies. We are advancing accordingly and plan to hold additional meetings with the FDA with the goal of achieving sufficient alignment to proceed on this basis and potentially resubmit our NDA for the treatment of liver fibrosis due to NASH in 2021. In addition, we continue to work collaboratively with the EMA on its review of our MAA and expect to submit our responses to the EMA's Day 120 questions in January 2021.

### ***Workforce Reduction***

On August 31, 2020, we adopted a plan to reduce our workforce in light of the previously announced receipt of a CRL from the FDA with respect to our NDA for OCA for the treatment of liver fibrosis due to NASH (the "2020 Workforce Plan"). The 2020 Workforce Plan seeks to streamline our operations and reduce operating expenses, while maintaining the critical resources needed to continue to support the NASH and PBC clinical programs, pursue the approval of OCA for the treatment of liver fibrosis due to NASH and support our successful PBC business. The 2020 Workforce Plan will result in a workforce reduction of approximately 25%, or approximately 170 employees. The 2020 Workforce Plan was implemented during the third quarter of 2020, immediately after its announcement, and is expected to be substantially completed by the end of 2020.

### ***COVID-19***

In March 2020, we announced new initiatives intended to ensure business continuity and support our employees during the evolving coronavirus ("COVID-19") pandemic, while continuing the critical activities necessary to bring our approved medicines to patients. With respect to our ongoing clinical trials, we are continuing to closely monitor the COVID-19 situation and together with our contract research organizations, study sites and other partners, have taken measures intended to minimize disruption to these studies. Our pivotal Phase 3 NASH trials, REGENERATE and REVERSE, are fully enrolled and our focus is to provide support to participating sites and patients including, where appropriate, through the use of telemedicine, home care visits, direct delivery of medication and other measures. With respect to our ongoing PBC trials, we paused screening and randomization in March 2020 in our Phase 4 COBALT confirmatory outcomes trial evaluating Ocaliva for PBC and our Phase 2 study evaluating bezafibrate in combination with OCA for PBC. We have now reinitiated screening and randomization in our COBALT trial and in our Phase 2 study evaluating bezafibrate in combination with OCA. With respect to our supply chain, we are working with our third-party manufacturers, distributors and other partners to manage our supply chain activities and mitigate disruptions to our product supplies. To protect the health of our employees and their families and communities, and in accordance with guidance issued by the U.S. Centers for Disease Control and Prevention, the World Health Organization and local authorities, our

global workforce is largely working remotely and we are leveraging digital communication technologies where appropriate to facilitate interactions with patients, healthcare professionals and our other stakeholders.

In addition, we are closely evaluating the impact of COVID-19 on our ability to effectively market, sell and distribute Ocaliva for PBC. Our field-based employees are often working remotely and are interacting with healthcare professionals via digital communication technologies such as, where appropriate, video conferences, emails and phone calls. Many of the healthcare professionals that we call on are working from home, facing additional demands on their time due to COVID-19, and may cancel diagnostic, elective, specialty and other procedures and appointments to avoid non-essential patient exposure to medical environments and potential infection with COVID-19. We are experiencing increased competition for virtual appointments with healthcare professionals. Our increased utilization of virtual interactions and the reduced quantity of such interactions during the COVID-19 pandemic may reduce the effectiveness of our sales personnel and negatively affect physician awareness and sales of Ocaliva. In the third quarter of 2020, we continued to see a lower level of prescriptions for new patients than we did prior to the outbreak of COVID-19. Although new patient starts currently account for only a small percentage of total Ocaliva prescriptions, our future sales of Ocaliva could be negatively impacted if prescriptions for new patients do not return to levels seen prior to the outbreak of COVID-19. It is also possible that COVID-19 may negatively affect our product sales in the future due to patient challenges in accessing healthcare providers, significant increases in unemployment and the resulting loss of individual health insurance coverage, and an inability to access government healthcare programs due to backlogs or other factors. COVID-19 could affect the operations of the FDA and/or negatively impact data capture and data quality of our ongoing clinical trials, which could delay or otherwise materially affect our clinical development efforts and the review and approval of our product candidates, including OCA for liver fibrosis due to NASH. Further, our ability to timely and cost-effectively resolve issues raised in the CRL to the satisfaction of the FDA may prove more difficult as a result of COVID-19. As public health restrictions on travel and in-person interactions are modified or relaxed, we may continue to face challenges that limit our ability to fully resume in-person interactions, including the potential for additional outbreaks, limited access to personal protective equipment, the need to navigate varying restrictions for entering healthcare facilities and employee illness and childcare obligations during school closures. We also expect that the conversion of medical conferences to a virtual format may reduce our ability to appropriately disseminate scientific information and conduct disease state education. The long-term effects of COVID-19 are unknown and it is possible that following the pandemic, healthcare institutions could alter their policies with respect to in-person visits by pharmaceutical company representatives.

The extent to which COVID-19 may impact our business will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the geographic spread of the disease, the duration of the outbreak, public health restrictions on travel and in-person interactions, business closures and disruptions, and the effectiveness of actions taken to contain and treat the disease. We cannot presently predict the duration, scope or severity of the potential effects of COVID-19 on our operations, including our clinical trials, regulatory submissions and reviews, supply chain or our ability to generate product sales from Ocaliva or, if approved, OCA for liver fibrosis due to NASH, and any such effects could have a material adverse impact on our business, results of operation and financial condition. See “—Risks Related to the Development and the Regulatory Review and Approval of Our Products and Product Candidates” and “—Risks Related to the Commercialization of Our Products” below.

## **Financial Overview**

### ***Revenue***

We commenced our commercial launch of Ocaliva for the treatment of PBC in the United States in June 2016. In December 2016, the European Commission granted conditional approval for Ocaliva for the treatment of PBC and we commenced our European commercial launch in January 2017. Since January 2017, Ocaliva has also received regulatory approval in several of our target markets outside the United States and Europe, including Canada, Israel and Australia. We sell Ocaliva to a limited number of specialty pharmacies which dispense the product directly to patients. The specialty pharmacies are referred to as our customers.

### *Product Revenue, Net*

We recognize revenue at the time of shipment to our customers on a sell-in basis. We provide the right of return to our customers for unopened product for a limited time before and after its expiration date.

Under Accounting Standards Codification (“ASC”) Topic 606, *Revenue from Contracts with Customers* (“ASC 606”), we have written contracts with each of our customers that have a single performance obligation — to deliver products upon receipt of a customer order — and these obligations are satisfied when delivery occurs and the customer receives Ocaliva. We evaluate the creditworthiness of each of our customers to determine whether collection is reasonably assured. We estimate variable revenue by calculating gross product revenues based on the wholesale acquisition cost that we charge our customers for Ocaliva, and then estimating our net product revenues by deducting (i) trade allowances, such as invoice discounts for prompt payment and customer fees, (ii) estimated government rebates and discounts related to Medicare, Medicaid and other government programs, and (iii) estimated costs of incentives offered to certain indirect customers including patients.

We recognized net sales of Ocaliva of \$79.5 million and \$61.5 million for the three months ended September 30, 2020 and 2019, respectively, and \$229.4 million and \$179.3 million for the nine months ended September 30, 2020 and 2019, respectively.

We have received paragraph IV certification notice letters from several generic drug manufacturers indicating that each company has submitted to the FDA an Abbreviated New Drug Application (“ANDA”) seeking approval to manufacture and sell a generic version of our 5 mg and 10 mg dosage strengths of Ocaliva® (obeticholic acid) for PBC prior to the expiration of certain patents protecting Ocaliva. We have initiated patent infringement suits against each of these generic drug manufacturers in the United States District Court for the District of Delaware. While we intend to vigorously defend and enforce our intellectual property rights protecting Ocaliva, we can offer no assurance as to when the lawsuits will be decided, whether the lawsuits will be successful, or that a generic equivalent of Ocaliva will not be approved and enter the market before the expiration of such patents. See Note 18 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q for more information.

### *Licensing Revenue*

In March 2011, we entered into an exclusive license agreement (the “Original Sumitomo Agreement”) with Sumitomo Dainippon Pharma Co., Ltd. (“Sumitomo Dainippon”), pursuant to which we granted to Sumitomo Dainippon an exclusive license to research, develop and commercialize OCA for the treatment of PBC and NASH in Japan and China (excluding Taiwan) and an option to research, develop and commercialize OCA in certain countries outside of such territories (the “Country Option”). We received an upfront payment from Sumitomo Dainippon of \$15.0 million under the terms of the Original Sumitomo Agreement. In October 2019, we and Sumitomo Dainippon mutually agreed to terminate with immediate effect the Sumitomo Agreement. In connection with the termination of the Sumitomo Agreement, Sumitomo Dainippon agreed to return to us the rights to develop and commercialize OCA in China and we agreed to forego any further milestone or royalty payments relating to the development and commercialization of OCA in China. No payment is due from us to Sumitomo Dainippon as a result of the termination of the Sumitomo Agreement.

The Company recognized licensing revenue of \$0 and \$0.4 million for the three months ended September 30, 2020 and 2019, respectively, and \$0 and \$1.2 million for the nine months ended September 30, 2020 and 2019, respectively. The licensing revenue in 2019 relates to the amortization of the upfront payments under the Sumitomo Agreement.

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

We have incurred and expect to continue to incur significant selling, general and administrative expenses as a result of, among other initiatives, the launch and commercialization of Ocaliva for PBC in the United States, Europe and our other target markets. In addition, we have incurred significant selling, general and administrative expenses and may in the future incur similar expenses in connection with the preparation for the potential commercialization of OCA for liver fibrosis due to NASH, if approved, and our other future approved products, if any, and the build-out of our general and administrative infrastructure in the United States and abroad.

**Research and Development Expenses**

Since our inception, we have focused significant resources on our research and development activities, including conducting preclinical studies and clinical trials, pursuing regulatory approvals and engaging in other product development activities. We recognize research and development expenses as they are incurred.

We have incurred and expect to continue to incur significant research and development expenses as a result of, among other initiatives, our clinical development programs for OCA for PBC and NASH, our other earlier stage research programs and our regulatory approval efforts.

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

The following table summarizes our results of operations for the three months ended September 30, 2020 and 2019:

	<b>Three Months Ended September 30,</b>	
	<b>2020</b>	<b>2019</b>
	<b>(in thousands)</b>	
<b>Revenue:</b>		
Product revenue, net	\$ 79,521	\$ 61,545
Licensing revenue	—	405
Total revenue	<u>79,521</u>	<u>61,950</u>
<b>Operating expenses:</b>		
Cost of sales	1,826	487
Selling, general and administrative	70,619	76,828
Research and development	48,858	60,168
Restructuring	13,381	—
Total operating expenses	<u>134,684</u>	<u>137,483</u>
<b>Other income (expense):</b>		
Interest expense	(12,091)	(11,795)
Other income, net	785	2,495
Total other (expense), net	<u>(11,306)</u>	<u>(9,300)</u>
Net loss	<u>\$ (66,469)</u>	<u>\$ (84,833)</u>

**Revenues**

Product revenue, net was \$79.5 million and \$61.5 million for the three months ended September 30, 2020 and 2019, respectively. For the three months ended September 30, 2020 and 2019, product revenue, net was comprised of U.S. Ocaliva net sales of \$58.6 million and \$45.2 million, respectively, and ex-U.S. Ocaliva net sales of \$20.9 million and \$16.3 million, respectively. We commenced our commercial launch of Ocaliva for the treatment of PBC in the United States and certain European countries in June 2016 and January 2017, respectively. Since January 2017, Ocaliva has also received regulatory approval in several of our target markets outside the United States and Europe, including Canada, Israel and Australia. For the three months ended September 30, 2020 and 2019, licensing revenue was \$0 and \$0.4 million, respectively. In the case of the three months ended September 30, 2019, revenues were related to the amortization of upfront payments under the Sumitomo Agreement.

**Cost of sales**

Cost of sales was \$1.8 million and \$0.5 million for the three months ended September 30, 2020 and 2019, respectively. Our cost of sales for the three months ended September 30, 2020 and 2019 consisted primarily of packaging, labeling, materials and related expenses.

*Selling, general and administrative expenses*

Selling, general and administrative expenses were \$70.6 million and \$76.8 million for the three months ended September 30, 2020 and 2019, respectively. The \$6.2 million net decrease between periods was primarily driven by reductions in spend resulting from the delay of the potential approval and commercialization of OCA for liver fibrosis due to NASH.

*Research and development expenses*

Research and development expenses were \$48.9 million and \$60.2 million for the three months ended September 30, 2020 and 2019, respectively. The \$11.3 million net decrease between periods was primarily driven by lower NASH development costs, including the conclusion of enrollment activities for the REGENERATE and REVERSE studies and reduced costs related to our preparations for regulatory interactions.

*Restructuring expenses*

Restructuring expenses were \$13.4 million and \$0 for the three months ended September 30, 2020 and 2019, respectively. The increase between periods was driven primarily by severance costs and other related termination benefits incurred in conjunction with the 2020 Workforce Plan.

*Interest expense*

Interest expense was \$12.1 million and \$11.8 million for the three months ended September 30, 2020 and 2019, respectively. For the three months ended September 30, 2020 and 2019, interest expense related to the \$230.0 million aggregate principal amount of 2.00% Convertible Senior Notes due 2026 (the “2026 Convertible Notes”) that we issued in May 2019 and the \$460.0 million aggregate principal amount of 3.25% Convertible Senior Notes due 2023 (the “2023 Convertible Notes”) and together with the 2026 Convertible Notes, the “Convertible Notes”) that we issued in July 2016.

*Other income, net*

Other income, net was \$0.8 million and \$2.5 million for the three months ended September 30, 2020 and 2019, respectively. Such income is primarily attributable to interest income earned on cash, cash equivalents and investment debt securities.

*Income taxes*

For the three months ended September 30, 2020 and 2019, no income tax expense or benefit was recognized. Our deferred tax assets are comprised primarily of net operating loss carryforwards. We maintain a full valuation allowance on our deferred tax assets since we have not yet achieved sustained profitable operations. As a result, we have not recorded any income tax benefit since our inception.

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

The following table summarizes our results of operations for the nine months ended September 30, 2020 and 2019:

	Nine Months Ended September 30,	
	2020	2019
	(in thousands)	
<b>Revenue:</b>		
Product revenue, net	\$ 229,422	\$ 179,286
Licensing revenue	—	1,216
Total revenue	<u>229,422</u>	<u>180,502</u>
<b>Operating expenses:</b>		
Cost of sales	4,555	1,738
Selling, general and administrative	262,537	223,738
Research and development	139,587	178,163
Restructuring	13,381	—
Total operating expenses	<u>420,060</u>	<u>403,639</u>
<b>Other income (expense):</b>		
Interest expense	(35,801)	(29,518)
Other income, net	3,706	6,132
Total other (expense), net	<u>(32,095)</u>	<u>(23,386)</u>
Net loss	<u>\$ (222,733)</u>	<u>\$ (246,523)</u>

**Revenues**

Product revenue, net was \$229.4 million and \$179.3 million for the nine months ended September 30, 2020 and 2019, respectively. For the nine months ended September 30, 2020 and 2019, product revenue, net was comprised of U.S. Ocaliva net sales of \$169.0 million and \$133.9 million, respectively, and ex-U.S. Ocaliva net sales of \$60.4 million and \$45.4 million, respectively. We commenced our commercial launch of Ocaliva for the treatment of PBC in the United States and certain European countries in June 2016 and January 2017, respectively. Since January 2017, Ocaliva has also received regulatory approval in several of our target markets outside the United States and Europe, including Canada, Israel and Australia. For the nine months ended September 30, 2020 and 2019, licensing revenue was \$0 and \$1.2 million, respectively. In the case of the nine months ended September 30, 2019, revenues were related to the amortization of upfront payments under the Sumitomo Agreement.

**Cost of sales**

Cost of sales was \$4.6 million and \$1.7 million for the nine months ended September 30, 2020 and 2019, respectively. Our cost of sales for the nine months ended September 30, 2020 and 2019 consisted primarily of packaging, labeling, materials and related expenses.

**Selling, general and administrative expenses**

Selling, general and administrative expenses were \$262.5 million and \$223.7 million for the nine months ended September 30, 2020 and 2019, respectively. The \$38.8 million net increase between periods was primarily driven by increases in expenses mostly in the first half of 2020 relating to our launch preparation activities associated with the potential approval and commercialization of OCA for liver fibrosis due to NASH.

**Research and development expenses**

Research and development expenses were \$139.6 million and \$178.2 million for the nine months ended September 30, 2020 and 2019, respectively. The \$38.6 million net decrease between periods was primarily driven by UK R&D tax

[Table of Contents](#)

credits recognized as a reduction of research and development expenses during the nine months ended September 30, 2020, lower NASH development costs resulting from the conclusion of enrollment activities for the REGENERATE and REVERSE studies and reduced costs related to the preparation for regulatory interactions.

*Restructuring expenses*

Restructuring expenses were \$13.4 million and \$0 for the nine months ended September 30, 2020 and 2019, respectively. The increase between periods was driven primarily by severance costs and other related termination benefits incurred in conjunction with the 2020 Workforce Plan.

*Interest expense*

Interest expense was \$35.8 million and \$29.5 million for the nine months ended September 30, 2020 and 2019, respectively. For the nine months ended September 30, 2020 and 2019, interest expense related to the 2026 Convertible Notes that we issued in May 2019 and the 2023 Convertible Notes that we issued in July 2016.

*Other income, net*

Other income, net was \$3.7 million and \$6.1 million for the nine months ended September 30, 2020 and 2019, respectively. Such income is primarily attributable to interest income earned on cash, cash equivalents and investment debt securities.

*Income taxes*

For the nine months ended September 30, 2020 and 2019, no income tax expense or benefit was recognized. Our deferred tax assets are comprised primarily of net operating loss carryforwards. We maintain a full valuation allowance on our deferred tax assets since we have not yet achieved sustained profitable operations. As a result, we have not recorded any income tax benefit since our inception.

**Liquidity and Capital Resources****Cash Flows**

The following table sets forth the significant sources and uses of cash for the periods indicated:

	<b>Nine Months Ended September 30,</b>	
	<b>2020</b>	<b>2019</b>
	<b>(in thousands)</b>	
Net cash (used in) provided by:		
Operating activities	\$ (154,051)	\$ (176,129)
Investing activities	132,973	(254,876)
Financing activities	(537)	451,639
Effect of exchange rate changes	(1,674)	(505)
Net (decrease) increase in cash, cash equivalents and restricted cash	<u>\$ (23,289)</u>	<u>\$ 20,129</u>

*Operating Activities.* Net cash used in operating activities of approximately \$154.1 million during the nine months ended September 30, 2020 was primarily a result of our \$222.7 million net loss and a net decrease in operating assets and liabilities of \$6.6 million, partially offset by \$44.4 million in stock-based compensation, \$12.3 million for accretion of the discount on the 2023 Convertible Notes, \$7.0 million for accretion of the discount on the 2026 Convertible Notes, \$4.6 million for non-cash operating lease costs and \$2.3 million of depreciation. Cash flows for the nine months ended September 30, 2020 include cash receipts of \$20.7 million reflecting payments from the HMRC for the U.K. R&D tax credit claims.

Net cash used in operating activities of approximately \$176.1 million during the nine months ended September 30, 2019 was primarily a result of our \$246.5 million net loss and a gain on lease termination of \$2.0 million, partially offset by \$42.8 million in stock-based compensation, \$15.1 million for accretion of the discounts on the Convertible Notes, a net increase in operating assets and liabilities of \$4.1 million, including a U.K. research & development tax credit of \$10.5 million, \$2.7 million for loss on the disposal of fixed assets, \$4.0 million for non-cash operating lease costs and \$2.8 million of depreciation.

*Investing Activities.* For the nine months ended September 30, 2020, net cash provided by investing activities primarily reflects the sales and maturities of investment debt securities of \$417.6 million, partially offset by the purchase of investment debt securities of \$282.6 million.

For the nine months ended September 30, 2019, net cash used in investing activities primarily reflects the purchase of investment debt securities of \$560.7 million, partially offset by the sales and maturities of investment debt securities of \$306.9 million.

*Financing Activities.* Net cash used in financing activities in the nine months ended September 30, 2020 consisted primarily of \$1.8 million from payments of employee withholding taxes related to stock-based awards offset by \$1.3 million of net proceeds from the exercise of options to purchase common stock.

Net cash provided by financing activities in the nine months ended September 30, 2019 consisted primarily of net proceeds received from the 2019 Public Offering and 2019 Concurrent Private Placement of \$227.2 million and net proceeds from the issuance of the 2026 Convertible Notes of \$223.4 million.

### **Future Funding Requirements**

As of September 30, 2020, we had \$496.8 million in cash, cash equivalents, restricted cash and investment debt securities. We currently expect to continue to incur significant operating expenses in the fiscal year ending December 31, 2020. These expenses are planned to support, among other initiatives, the continued commercialization of Ocaliva for PBC in the United States and our other markets, our continued clinical development of OCA for PBC and NASH and our other earlier stage research and development programs. Although we believe that our existing capital resources, together with our net sales of Ocaliva for PBC, will be sufficient to fund our anticipated operating requirements for the next twelve months following the filing of this report, we may need to raise additional capital to fund our operating requirements beyond that period. Furthermore, in light of our receipt of the CRL from the FDA in June 2020 with respect to our NDA for OCA for liver fibrosis due to NASH and the numerous risks and uncertainties associated with pharmaceutical product development and commercialization, any delays in, or unanticipated costs associated with, our development, regulatory or commercialization efforts could significantly increase the amount of capital required by us to fund our operating requirements. Accordingly, we may seek to access the public or private capital markets whenever conditions are favorable, even if we do not have an immediate need for additional capital at that time.

Our forecasts regarding the period of time that our existing capital resources will be sufficient to meet our operating requirements and the timing of our future funding requirements, both near and long-term, will depend on a variety of factors, many of which are outside of our control. Such factors include, but are not limited to:

- our ability to successfully commercialize Ocaliva for PBC;
- our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization;
- our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL by the FDA; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; or any future determination that the regulatory applications and subsequent

information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval;

- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of any of our products or product candidates;
- any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions or otherwise limit the sale of such product or product candidate;
- the initiation, timing, cost, conduct, progress and results of our research and development activities, preclinical studies and clinical trials, including any issues, delays or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials;
- our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our clinical trial activities;
- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to NASH, if approved;
- our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend and enforce any patent claims or other intellectual property rights;
- the size and growth of the markets for our products and product candidates and our ability to serve those markets;
- the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH or our other product candidates among physicians, patients and healthcare payors;
- the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products;
- our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties;
- competition from existing drugs or new drugs that become available;
- our ability to prevent system failures, data breaches or violations of data protection laws;
- costs and outcomes relating to any disputes, governmental inquiries or investigations, regulatory proceedings, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation;
- our collaborators' election to pursue research, development and commercialization activities;

- our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise;
- our need for and ability to generate or obtain additional financing;
- our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof;
- our use of cash and short-term investments;
- our ability to acquire, license and invest in businesses, technologies, product candidates and products;
- our ability to attract and retain key personnel to manage our business effectively;
- our ability to manage the growth of our operations, infrastructure, personnel, systems and controls;
- our ability to obtain and maintain adequate insurance coverage;
- the impact of COVID-19, including any impact on our results of operations or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof;
- the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and
- the other risks and uncertainties identified under the captions “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Quarterly Report on Form 10-Q and in our other periodic filings filed with the SEC, including our Annual Report on Form 10-K for the year ended December 31, 2019.

We have no committed external sources of funding and additional funds may not be available when we need them on terms that are acceptable to us, or at all. In addition, in recent months global markets have experienced significant volatility in connection with concerns over the impact of COVID-19, and such concerns may in the future materially and adversely affect our ability to raise funds. If adequate funds are not available to us, we may not be able to make scheduled debt payments on a timely basis, or at all, and may be required to delay, limit, reduce or cease our operations.

### **Contractual Obligations**

There have been no material changes to our contractual obligations outside the ordinary course of business from those disclosed under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Contractual Obligations” in our Annual Report on Form 10-K for the year ended December 31, 2019.

### **Off-Balance Sheet Arrangements**

As of September 30, 2020, we did not have any off-balance sheet arrangements.

### **Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates and there have been no material changes to our market risk from that disclosed under the caption “Quantitative and Qualitative Disclosures about Market Risk” in our Annual Report.

**Item 4. Controls and Procedures.**

**Evaluation of Disclosure Controls and Procedures**

Based on the evaluation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), required by Rule 13a-15(b) or 15d-15(b) of the Exchange Act, our Chief Executive Officer and Chief Financial Officer have concluded that as of the end of the period covered by this Quarterly Report on Form 10-Q, our disclosure controls and procedures were effective.

**Changes in Internal Control Over Financial Reporting**

There were no changes in our internal control over financial reporting that occurred during our most recent fiscal quarter 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.

For a description of our significant legal proceedings, see Note 18 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q and incorporated by reference herein.

### Item 1A. Risk Factors.

*Investing in our securities involves a high degree of risk. The following risk factors and other information included in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2019 should be carefully considered before deciding whether to invest in our securities. The risks and uncertainties described below and in our other filings are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. If any of the following risks, or such unknown risks, occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In that case, the market price of our securities could decline, and you may lose all or part of your investment.*

#### **Risks Related to Our Financial Position and Need for Additional Capital**

***We are currently dependent on the successful commercialization of Ocaliva for PBC. To the extent Ocaliva is not commercially successful, our business, financial condition and results of operations may be materially and adversely affected and the price of our common stock may decline.***

Ocaliva is our only drug that has been approved for sale and it has only been approved for the treatment of PBC in combination with UDCA in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA.

Our ability to generate profits from operations and become profitable currently depends on the commercial success of Ocaliva for PBC. However, the successful commercialization of Ocaliva for PBC is subject to many risks. We have not launched or commercialized a drug before Ocaliva, and there is no guarantee that we will be able to do so successfully. There are numerous examples of unsuccessful product launches and commercial efforts, as well as failures to meet expectations of market potential, including by pharmaceutical companies with greater experience and resources than us.

The commercial success of Ocaliva for PBC depends on the extent to which patients, physicians and payers accept and adopt Ocaliva as a treatment for PBC, and we do not know whether our or others' estimates in this regard will be accurate. As such, there is significant uncertainty in the degree of market acceptance that Ocaliva will have for PBC. For example, if the patient population suffering from PBC is smaller than we estimate, or even if the patient population matches our estimates but Ocaliva is not widely accepted as a treatment for PBC, the commercial potential of Ocaliva for PBC will be limited. Physicians may not prescribe Ocaliva and patients may be unwilling to use Ocaliva if coverage is not provided or reimbursement is inadequate to cover a significant portion of the cost. In the third quarter of 2020, we continued to see a lower level of prescriptions for new patients than we did prior to the outbreak of COVID-19 and related government-imposed shelter-in-place mandates, restrictions on in-person interactions and other public health safety measures. The future impact of COVID-19, the governmental responses thereto and the resulting rate of prescriptions for new patients remains difficult to predict. If the rate of prescriptions for new patients declines in the future, we may see a reduction in net sales of Ocaliva, which could negatively affect our business, financial condition and results of operations. Additionally, the use of Ocaliva in a non-trial setting may result in the occurrence of unexpected or a greater incidence of side effects, adverse reactions or misuse that may negatively affect the commercial prospects of Ocaliva for PBC. Furthermore, any negative development in any other development program for OCA or our failure to satisfy the post-marketing regulatory commitments and requirements to which we are or may become subject, including the completion of our Phase 4 COBALT trial, may materially and adversely impact the commercial results and potential of Ocaliva for PBC. See "—Risks Related

to the Development and the Regulatory Review and Approval of Our Products and Product Candidates” and “—Risks Related to the Commercialization of Our Products” below.

As a result, it is uncertain whether Ocaliva net sales for PBC will sustain our operations and it may take a significant amount of time before Ocaliva net sales for PBC sustain our operations. Furthermore, Ocaliva may not receive regulatory approval for PBC in jurisdictions beyond those in which it is currently approved, which may also limit our prospects. If the commercialization of Ocaliva for PBC is unsuccessful or perceived to be unsuccessful, the long-term prospects of Ocaliva for PBC, as well as the long-term prospects of our company, may be materially and adversely affected.

***We have never been profitable. We expect to incur losses for the foreseeable future, and we may never achieve or sustain profitability.***

We have never been profitable and do not expect to be profitable in the foreseeable future. We incurred net losses of \$344.7 million, \$309.2 million and \$360.4 million for the years ended December 31, 2019, 2018 and 2017, respectively. To date, we have financed our operations primarily through public offerings and private placements of our securities, sales of product and payments received under licensing and collaboration agreements. At September 30, 2020, we had \$496.8 million in cash, cash equivalents, restricted cash and investment debt securities.

We have devoted substantially all of our resources to the development of our product candidates, including the conduct of our clinical trials, the launch and commercialization of Ocaliva for PBC, preparation for a potential launch of OCA for liver fibrosis due to NASH and general and administrative operations, including the protection of our intellectual property.

We expect to continue to incur losses for the foreseeable future, and we expect these losses to be significant as we, among other things, develop and seek regulatory approval for our product candidates, including OCA for liver fibrosis due to NASH, maintain our regulatory approvals and commercialize our approved products. We believe our prospects and ability to significantly grow revenues will be dependent on our ability to successfully develop and commercialize OCA for indications other than PBC, such as NASH. As a result, we expect a significant amount of resources to continue to be devoted to our development programs for OCA.

As part of our product development activities, we currently expect to continue our Phase 3 clinical program of OCA for liver fibrosis due to NASH, including our Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH through clinical outcomes for verification and description of clinical benefit and our Phase 3 REVERSE trial for NASH patients with compensated cirrhosis. Our expenses could increase if we are required by the FDA or the EMA to perform studies or trials in addition to those currently expected, if our current trials are modified for any reason, or if there are any issues or delays in completing our clinical trials or the development of any of our product candidates, due to COVID-19 or otherwise. For example, in June 2020 we received a CRL from the FDA with respect to our NDA for OCA for liver fibrosis due to NASH. The CRL indicated that, based on the data the FDA had reviewed, the FDA has determined that the predicted benefit of OCA based on a surrogate histopathologic endpoint remains uncertain and does not sufficiently outweigh the potential risks to support accelerated approval for the treatment of patients with liver fibrosis due to NASH. The FDA recommended that we submit additional post-interim analysis efficacy and safety data from the ongoing REGENERATE trial in support of potential accelerated approval and that the long-term outcomes phase of the trial should continue. Although we are in discussions with the FDA with respect to the potential resubmission of our NDA seeking accelerated approval of OCA for the treatment of liver fibrosis due to NASH, there is no assurance that we will be successful or that OCA will be approved for liver fibrosis due to NASH on an accelerated basis, or at all. Accordingly, our previously anticipated U.S. commercial launch of OCA for liver fibrosis due to NASH has been postponed, we do not expect to generate revenues for this indication until it has been approved, and we may incur significantly greater costs than previously anticipated in connection with the development of OCA for liver fibrosis due to NASH.

We intend to continue to develop OCA and our other existing product candidates, alone or in combination, for non-viral liver diseases. While we paused screening and randomization in March 2020 in our Phase 4 COBALT confirmatory outcomes trial evaluating Ocaliva for PBC and our Phase 2 study evaluating bezafibrate in combination with OCA for PBC due to COVID-19, we have now reinitiated screening and randomization in these trials. If OCA or any of our other

product candidates fails in clinical trials or does not gain or maintain regulatory approval, or if OCA or any of our other product candidates does not achieve market acceptance, we may never become profitable. Our net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. Because of the numerous risks and uncertainties associated with pharmaceutical product development and commercialization, we are unable to predict with certainty the timing or amount of our expenses, whether such expenses may increase, or when, or if, we will be able to achieve profitability. The amount of our future net losses will depend, in part, on our future expenses, whether and by how much such expenses increase and our ability to generate revenues.

***We will require substantial additional funding, which may not be available to us on acceptable terms, if at all. If adequate funds are not available to us, we may be required to delay, limit, reduce or cease our operations.***

We are currently developing OCA for additional indications, including NASH, and other product candidates through various stages of clinical and preclinical development. Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is expensive. If, for example, the FDA, EMA or other regulatory authorities require that we perform additional studies beyond those that we currently expect, our expenses could increase materially beyond what we currently anticipate, and the timing of any potential product approval may be delayed.

In addition, we have incurred and anticipate that we will continue to incur significant research and development, product sales, marketing, manufacturing and distribution expenses relating to the commercialization of Ocaliva for PBC. As part of our longer-term strategy, we anticipate that we will incur significant expenses in connection with our research and development efforts, the commercialization of our other products such as OCA for liver fibrosis due to NASH, if approved, and the build-out of our general and administrative infrastructure in the United States and abroad. We may also engage in business development activities that involve potential in- or out-licensing of products or technologies or acquisitions of other products, technologies or businesses.

As of September 30, 2020, we had \$496.8 million in cash, cash equivalents, restricted cash and investment debt securities. We currently expect to continue to incur significant operating expenses in the fiscal year ending December 31, 2020. These expenses are planned to support, among other initiatives, the continued commercialization of Ocaliva for PBC in the United States and our other markets, our continued clinical development of OCA for PBC and NASH and our other earlier stage research and development programs. Although we believe that our existing capital resources, together with our net sales of Ocaliva for PBC, will be sufficient to fund our anticipated operating requirements for the next twelve months, we may need to raise additional capital to fund our operating requirements beyond that period. Furthermore, in light of our recent receipt of a CRL from the FDA with respect to our NDA for OCA for liver fibrosis due to NASH and the numerous risks and uncertainties associated with pharmaceutical product development and commercialization, any delays in, or unanticipated costs associated with, our development, regulatory or commercialization efforts could significantly increase the amount of capital required by us to fund our operating requirements. Accordingly, we may seek to access the public or private capital markets whenever conditions are favorable, even if we do not have an immediate need for additional capital at that time.

Our forecasts regarding the period of time that our existing capital resources will be sufficient to meet our operating requirements and the timing of our future funding requirements, both near and long-term, will depend on a variety of factors, many of which are outside of our control. Such factors include, but are not limited to:

- our ability to successfully commercialize Ocaliva for PBC;
- our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization;
- our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL by the FDA; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; or any future determination that the regulatory applications and subsequent

information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval;

- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of any of our products or product candidates;
- any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions or otherwise limit the sale of such product or product candidate;
- the initiation, timing, cost, conduct, progress and results of our research and development activities, preclinical studies and clinical trials, including any issues, delays or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials;
- our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our clinical trial activities;
- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to NASH, if approved;
- our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend and enforce any patent claims or other intellectual property rights;
- the size and growth of the markets for our products and product candidates and our ability to serve those markets;
- the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH or our other product candidates among physicians, patients and healthcare payors;
- the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products;
- our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties;
- competition from existing drugs or new drugs that become available;
- our ability to prevent system failures, data breaches or violations of data protection laws;
- costs and outcomes relating to any disputes, governmental inquiries or investigations, regulatory proceedings, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation;
- our collaborators' election to pursue research, development and commercialization activities;

## [Table of Contents](#)

- our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise;
- our need for and ability to generate or obtain additional financing;
- our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof;
- our use of cash and short-term investments;
- our ability to acquire, license and invest in businesses, technologies, product candidates and products;
- our ability to attract and retain key personnel to manage our business effectively;
- our ability to manage the growth of our operations, infrastructure, personnel, systems and controls;
- our ability to obtain and maintain adequate insurance coverage;
- the impact of COVID-19, including any impact on our results of operations or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof;
- the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and
- the other risks and uncertainties identified under the captions “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Quarterly Report on Form 10-Q and in our other periodic filings filed with the SEC, including our Annual Report on Form 10-K for the year ended December 31, 2019.

We have no committed external sources of funding and additional funds may not be available when we need them on terms that are acceptable to us, or at all. In addition, in recent months global markets have experienced significant volatility in connection with concerns over the impact of COVID-19, and such concerns may in the future materially and adversely affect our ability to raise funds. If adequate funds are not available to us, we may not be able to make scheduled debt payments on a timely basis, or at all, and may be required to delay, limit, reduce or cease our operations.

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

Unless and until we generate sufficient cash flow from sales of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, we expect to finance our future cash needs through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements or other collaborations, strategic alliances and licensing arrangements, or a combination of these sources. Additional funding may not be available to us on acceptable terms, if at all.

The terms of any future financing may adversely affect the interests of our existing securityholders. For example, to the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders’ ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. We also could be required to seek funds through arrangements with licensing or collaborative partners or otherwise that may require us to relinquish rights to some of our technologies or product

candidates or otherwise agree to terms unfavorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

***We have a limited operating history as a commercial organization, which may make it difficult to predict our future performance, and we expect to continue to face a number of factors that may cause operating results to fluctuate.***

We are a biopharmaceutical company with a limited operating history as a commercial organization. Prior to the launch and commercialization of Ocaliva for PBC, our operations were limited to developing our technology, undertaking preclinical studies and clinical trials of our product candidates and preparing for the commercial launch of Ocaliva for PBC. Other than Ocaliva for PBC, none of our other product candidates have received regulatory approval. Consequently, any predictions regarding our future success or viability may not be as accurate as they could be if we had a longer operating history or greater experience commercializing approved products.

The commercialization of Ocaliva for PBC has been and will continue to be, and, if approved, the commercialization of OCA for liver fibrosis due to NASH will be, expensive and time-consuming, and we cannot be certain that we will be able to generate sufficient revenues from sales of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH in our target markets to offset such costs. Furthermore, our financial condition and operating results have varied significantly in the past and are expected to continue to significantly fluctuate from quarter-to-quarter and year-to-year due to a variety of factors, many of which are outside of our control. Such factors include, but are not limited to:

- our ability to successfully commercialize Ocaliva for PBC;
- our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization;
- our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL by the FDA; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; or any future determination that the regulatory applications and subsequent information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval;
- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of any of our products or product candidates;
- any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions or otherwise limit the sale of such product or product candidate;
- the initiation, timing, cost, conduct, progress and results of our research and development activities, preclinical studies and clinical trials, including any issues, delays or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials;
- our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our clinical trial activities;

## [Table of Contents](#)

- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to NASH, if approved;
- our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend and enforce any patent claims or other intellectual property rights;
- the size and growth of the markets for our products and product candidates and our ability to serve those markets;
- the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH or our other product candidates among physicians, patients and healthcare payors;
- the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products;
- our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties;
- competition from existing drugs or new drugs that become available;
- our ability to prevent system failures, data breaches or violations of data protection laws;
- costs and outcomes relating to any disputes, governmental inquiries or investigations, regulatory proceedings, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation;
- our collaborators' election to pursue research, development and commercialization activities;
- our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise;
- our need for and ability to generate or obtain additional financing;
- our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof;
- our use of cash and short-term investments;
- our ability to acquire, license and invest in businesses, technologies, product candidates and products;
- our ability to attract and retain key personnel to manage our business effectively;
- our ability to manage the growth of our operations, infrastructure, personnel, systems and controls;
- our ability to obtain and maintain adequate insurance coverage;

- the impact of COVID-19, including any impact on our results of operations or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof;
- the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and
- the other risks and uncertainties identified under the captions “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Quarterly Report on Form 10-Q and in our other periodic filings filed with the SEC, including our Annual Report on Form 10-K for the year ended December 31, 2019.

#### **Risks Related to the Development and the Regulatory Review and Approval of Our Products and Product Candidates**

***We cannot be certain whether Ocaliva will receive full approval for PBC in jurisdictions where it has previously received accelerated or conditional approval, or that Ocaliva will be approved for PBC in any jurisdictions beyond those in which it is currently approved. Furthermore, OCA may not be approved on an accelerated basis, or at all, for NASH or any other indication beyond PBC and we may not receive regulatory approval for any other product candidate. Without regulatory approval, we will not be able to market and commercialize our product candidates.***

The development, testing, manufacture, packaging, labeling, storage, approval, promotion, advertising, distribution, marketing and export and import, among other things, of our products and product candidates are subject to extensive regulation by the FDA in the United States, the EMA in Europe and various regulatory authorities in other countries, with regulations differing from country to country. We are not permitted to market our product candidates in the United States or Europe until we receive approval of a NDA, from the FDA, or a MAA, from the EMA, respectively. Currently, our ability to generate product sales depends on the successful marketing of Ocaliva for PBC in the jurisdictions in which it has received regulatory approval. In the future, our ability to generate product sales in addition to those of Ocaliva for PBC will depend on whether we are successful in obtaining regulatory approval of our other product candidates, including OCA for liver fibrosis due to NASH.

Ocaliva is our only drug that has been approved for sale and it has only been approved for the treatment of PBC in combination with UDCA in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA. In the United States, Ocaliva was approved for PBC under the accelerated approval pathway. Accelerated approval was granted for Ocaliva for PBC based on a reduction in ALP; however, an improvement in survival or disease-related symptoms has not yet been established. Continued approval of Ocaliva for PBC in the United States is contingent upon the verification and description of clinical benefit in confirmatory trials and our satisfaction of our other post-marketing regulatory requirements. Due to COVID-19, we paused screening and randomization in our Phase 4 COBALT confirmatory outcomes trial and took measures intended to minimize disruptions and protect and retain enrolled patients participating in the ongoing trial. While we have recommenced enrollment in our COBALT trial, any delay of the trial or failure by us to confirm the clinical benefit of Ocaliva for PBC due to COVID-19 or other factors may jeopardize the continued approval of Ocaliva for PBC. As specified by the applicable post-marketing requirements, our COBALT trial includes patients across the spectrum of PBC disease, including early and advanced PBC. We have agreed to evaluate the safety and efficacy of Ocaliva in patients with moderate to severe hepatic impairment and as a monotherapy in patients with PBC. In addition, we agreed to develop and characterize a lower dose formulation of Ocaliva to allow for once daily dosing in patients with moderate or advanced hepatic impairment, and we have fulfilled this commitment.

We commenced our commercial launch of Ocaliva for PBC in certain European countries in 2017 following the European Commission’s grant of conditional approval in December 2016. Our marketing authorization in the European Union is conditioned on the successful completion of the COBALT trial and a trial evaluating the safety and efficacy of Ocaliva in patients with moderate to severe hepatic impairment.

Since January 2017, Ocaliva has also received regulatory approval in several of our target markets outside the United States and Europe, including Canada, Israel and Australia, and we are pursuing marketing approval of Ocaliva for PBC in our other international target markets. If obtained, continued approval of Ocaliva for PBC in such jurisdictions may be contingent upon the verification and description of clinical benefit in confirmatory trials. Any delay or failure in satisfying the post-marketing regulatory commitments and requirements to which we are or may become subject, including our COBALT trial, may jeopardize the continued approval of Ocaliva for PBC in the United States, European Union and other jurisdictions.

While we remain blinded to safety and efficacy data in the ongoing COBALT trial, a data monitoring committee recently reviewed the unblinded results of a pre-specified interim efficacy analysis, including unblinded safety data, pursuant to the study protocol. Though no acute safety concerns were observed by the data monitoring committee based on its review of the unblinded safety data and no changes to the conduct of the trial were recommended based on safety or otherwise, the data monitoring committee did issue a recommendation that it does not seem feasible to continue the COBALT trial as designed. As is typical based on the importance of maintaining data integrity in such situations, there was minimal additional detail provided by the data monitoring committee. Given the inherent challenges in enrolling and maintaining placebo-controlled post-marketing studies in a rare disease setting and the potential confounding impact of subjects discontinuing treatment and/or transitioning from investigational product to commercial drug during clinical trials, we have previously discussed with the FDA and the EMA the merits of potentially converting COBALT to an open label external control design, and both agencies advised that we revisit this possibility with them if warranted after the pre-specified interim efficacy analysis by the data monitoring committee. We have notified the FDA and the EMA of the data monitoring committee's recommendation and, as they previously advised, plan on meeting with the FDA to discuss the matter and seeking formal EU scientific advice with respect to potential alternative study designs. If we are unable to satisfactorily address the data monitoring committee's recommendation with the FDA and/or the EMA and the COBALT trial cannot be timely completed, cannot be completed as designed or fails, we may not be able to maintain our previously granted marketing approval of Ocaliva for PBC.

Ocaliva is not approved for any indication other than PBC. We currently have no other products approved for sale and we cannot guarantee that we will ever have additional marketable products or that OCA will be approved for use in additional indications such as NASH. NDAs and MAAs must include extensive preclinical and clinical data and supporting information to establish the product candidate's safety and effectiveness for each desired indication. NDAs and MAAs must also include significant information regarding the chemistry, manufacturing and controls for the product. Obtaining approval of a NDA or a MAA is a lengthy, expensive and uncertain process, and we may not be successful in obtaining approval. The FDA and the EMA review processes can take years to complete and approval is not guaranteed. Even after the submission of a NDA, the FDA may decide not to accept the submission for filing and review or may determine that the submission does not support approval. For example, in June 2020 we received a CRL from the FDA with respect to our NDA for OCA for liver fibrosis due to NASH. The CRL indicated that, based on the data the FDA had reviewed, the FDA had determined that the predicted benefit of OCA based on a surrogate histopathologic endpoint remains uncertain and does not sufficiently outweigh the potential risks to support accelerated approval for the treatment of patients with liver fibrosis due to NASH. The FDA recommended that we submit additional post-interim analysis efficacy and safety data from the ongoing REGENERATE study in support of potential accelerated approval and that the long-term outcomes phase of the study should continue. Similarly, there are a number of factors that may result in delays in the EMA's review process following the submission of a MAA, or the EMA may determine that the submission does not support approval on a conditional basis, or at all. The United Kingdom left the European Union on January 31, 2020, in what is often referred to as "Brexit". Because a significant proportion of the regulatory framework in the United Kingdom is currently derived from European Union directives and regulations, Brexit could (following the end of the Brexit transition period on December 31, 2020) result in material changes to the regulatory regime applicable to many of our current operations, including those relating to Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH. COVID-19 could also affect the operations of the FDA, EMA and other health authorities, which could delay our clinical development efforts and the review and approval of our product candidates, including OCA for liver fibrosis due to NASH.

As is the case with the approval of Ocaliva for PBC, any future approvals or potential future approvals may also be conditional upon the completion of one or more clinical trials. In addition, delays in approvals or rejections of marketing applications in the United States, Europe or other countries may be based upon many factors, including, for example, regulatory requests for additional analyses, reports, data, preclinical studies and clinical trials, regulatory endpoint

requirements, regulatory questions regarding safety or risk-benefit profile, different interpretations of data and results, changes in regulatory policy during the period of product development and the emergence of new information regarding our product candidates or approved products. Initial and continued regulatory approval is also dependent on successfully passing regulatory inspection requirements applicable to us, our clinical sites and our key vendors, including requirements that we and such parties comply with applicable good clinical, pharmacovigilance, laboratory and manufacturing practices regulations. Critical findings could jeopardize or delay the approval of our NDAs or MAAs or impair our ability to maintain our marketing approvals.

Prior to receiving regulatory approval, we must finalize the product label for each of our product candidates in each jurisdiction in which we seek regulatory approval. Even if our product is approved, the FDA, EMA or other applicable regulatory authorities may limit the indications or uses for which our product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials, risk mitigation programs such as a REMS, monitoring or reporting as a condition of approval. Also, regulatory approval for our approved products may be withdrawn. In addition, obtaining regulatory approval for the marketing of our product in one country does not ensure that we will be able to obtain regulatory approval for such product in any other country.

In order to obtain and/or maintain regulatory approval for OCA for indications other than PBC, we will need to complete additional clinical trials and studies. For example, in connection with our Phase 3 clinical program of OCA for liver fibrosis due to NASH, we are currently conducting our Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH through clinical outcomes for verification and description of clinical benefit and our Phase 3 REVERSE trial for NASH patients with compensated cirrhosis. While our ongoing Phase 3 REGENERATE and REVERSE trials are fully enrolled and we have taken measures intended to minimize disruptions and protect and retain patients participating in such trials due to COVID-19, we cannot predict the degree to which such measures will ultimately prove effective. Our ability to obtain and maintain the regulatory approvals necessary to commercialize OCA for indications other than PBC, including NASH, will depend on our ability to successfully design, conduct and complete these trials, the efficacy, safety and risk-benefit profile of OCA demonstrated by such trials and our ability to prepare and submit complex regulatory filings in accordance with applicable regulatory requirements.

There can be no assurance that OCA will receive marketing approval on an accelerated or conditional basis, or at all, for PBC in jurisdictions where it has not yet been approved or for NASH in any jurisdiction, or that any of our other product candidates will receive marketing approval for any indication in any jurisdiction. We cannot predict whether our clinical trials and studies for our product candidates, including OCA for PBC, NASH or any other indication, will be successful, whether regulatory authorities will agree with our conclusions relating to the clinical trials and studies we conduct, or whether such regulatory authorities will require us to conduct additional clinical trials or studies. For example, while OCA received breakthrough therapy designation from the FDA in January 2015 for the treatment of NASH patients with liver fibrosis and we filed a NDA in the United States and a MAA in Europe based on the results from the 18-month analysis of our Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH, we do not know if one pivotal clinical trial will be sufficient for marketing approval or if regulatory authorities in the United States, Europe or our other target markets will approve OCA for liver fibrosis due to NASH on an accelerated or conditional basis, or at all. Our Phase 3 REGENERATE trial remains blinded after the interim analysis and is expected to continue to follow patients until the occurrence of a pre-specified number of adverse clinical outcomes, including progression to cirrhosis, for verification and description of clinical benefit.

If we are unable to obtain regulatory approval for OCA for PBC in the jurisdictions in which it is not currently approved or obtain regulatory approval in the United States, European Union and other jurisdictions for OCA for other indications, such as NASH, or for our other product candidates, we may not be able to generate sufficient revenue to become profitable or to continue our operations.

***We are developing product candidates for the treatment of rare diseases or diseases for which there are no or limited therapies, such as PBC and NASH, and for some of which there is little clinical experience, and our development approach involves new endpoints and methodologies. As a result, there is a heightened risk that we will not be able to gain agreement with regulatory authorities regarding an acceptable development plan, that the outcome of our clinical trials will not be favorable or that, even if favorable, regulatory authorities may not find the results of our clinical trials to be sufficient for marketing approval.***

We are focused on developing therapeutics for the treatment of rare diseases and diseases for which there are no or limited treatments. As a result, the design and conduct of our clinical trials for these indications is subject to heightened risk.

In the United States, the FDA generally requires two adequate and well-controlled pivotal clinical trials to approve a NDA. Furthermore, for full approval of a NDA, the FDA requires a demonstration of efficacy based on a clinical benefit endpoint. The FDA may grant accelerated approval based on a surrogate endpoint reasonably likely to predict clinical benefit. Even though our pivotal clinical trials for a specific indication, such as our Phase 3 REGENERATE trial of OCA in patients with liver fibrosis due to NASH and our Phase 3 REVERSE trial for NASH patients with compensated cirrhosis, may achieve their primary endpoints and are reasonably believed by us to be likely to predict clinical benefit, the FDA may not accept the results of such trials or approve our product candidate on an accelerated basis, or at all. It is also possible that the FDA may refuse to accept for filing and review any regulatory application we submit for regulatory approval in the United States. Even if our regulatory application is accepted for review, there may be delays in the FDA's review process and the FDA may determine that such regulatory application does not contain adequate clinical or other data or support the approval of the product candidate. In such a case, the FDA may issue a CRL that may require that we conduct and/or complete additional clinical trials and preclinical studies or provide additional information or data before it will reconsider our application for approval. For example, in June 2020 we received a CRL from the FDA regarding our NDA for OCA for liver fibrosis due to NASH. The CRL indicated that, based on the data the FDA had reviewed, the FDA had determined that the predicted benefit of OCA based on a surrogate histopathologic endpoint remains uncertain and does not sufficiently outweigh the potential risks to support accelerated approval for the treatment of patients with liver fibrosis due to NASH. The FDA recommended that we submit additional post-interim analysis efficacy and safety data from the ongoing REGENERATE study in support of potential accelerated approval and that the long-term outcomes phase of the study should continue. The requirements imposed by the FDA may be substantial, expensive and time-consuming, and there is no guarantee that we will continue to pursue any such application or that the FDA will ultimately decide that any such application supports the approval of the product candidate on an accelerated basis, or at all. The FDA may also refer any regulatory application to an advisory committee for review and recommendation as to whether, and under what conditions, the application should be approved. While the FDA is not bound by the recommendation of an advisory committee, it considers such recommendations carefully when making decisions.

Even if we receive accelerated approval for any of our product candidates, we may be required to conduct or complete a post-approval clinical outcomes trial to confirm the clinical benefit of such product candidates by demonstrating the correlation of the surrogate endpoint therapeutic response in patients with a significant reduction in adverse clinical outcomes over time. For example, interim analysis results at 18 months in our Phase 3 REGENERATE trial were based on surrogate endpoints and the impact on clinical outcomes has not been confirmed. The REGENERATE trial is ongoing for verification and description of clinical benefit of OCA for liver fibrosis due to NASH. There can be no assurance that the clinical outcomes portion of our REGENERATE trial will confirm that the surrogate endpoint used as the basis of the regulatory submissions we have made or expect to make seeking approval of OCA for liver fibrosis due to NASH will eventually show an adequate correlation with clinical outcomes. In addition, as a condition of the accelerated approval of Ocaliva for PBC in the United States, we are required to conduct a clinical outcomes study with respect to Ocaliva for PBC. Following discussions with regulatory authorities, we initiated our COBALT clinical outcomes confirmatory trial for PBC in December 2014 prior to the approval of Ocaliva for PBC. The COBALT trial includes subjects across the spectrum of PBC disease, including early and advanced PBC. We have also agreed to evaluate the safety and efficacy of Ocaliva in patients with moderate to severe hepatic impairment and as a monotherapy in patients with PBC. There can be no assurance that our COBALT trial conducted as part of our post-marketing obligations will confirm that the surrogate

endpoint used for accelerated approval of Ocaliva for PBC will eventually show an adequate correlation with clinical outcomes or that our clinical trial in PBC patients with moderate to severe hepatic impairment will be successful.

While we remain blinded to safety and efficacy data in the ongoing COBALT trial, a data monitoring committee recently reviewed the unblinded results of a pre-specified interim efficacy analysis, including unblinded safety data, pursuant to the study protocol. Though no acute safety concerns were observed by the data monitoring committee based on its review of the unblinded safety data and no changes to the conduct of the trial were recommended based on safety or otherwise, the data monitoring committee did issue a recommendation that it does not seem feasible to continue the COBALT trial as designed. As is typical based on the importance of maintaining data integrity in such situations, there was minimal additional detail provided by the data monitoring committee. Given the inherent challenges in enrolling and maintaining placebo-controlled post-marketing studies in a rare disease setting and the potential confounding impact of subjects discontinuing treatment and/or transitioning from investigational product to commercial drug during clinical trials, we have previously discussed with the FDA and the EMA the merits of potentially converting COBALT to an open label external control design, and both agencies advised that we revisit this possibility with them if warranted after the pre-specified interim efficacy analysis by the data monitoring committee. We have notified the FDA and the EMA of the data monitoring committee's recommendation and, as they previously advised, plan on meeting with the FDA to discuss the matter and seeking formal EU scientific advice with respect to potential alternative study designs. If we are unable to satisfactorily address the data monitoring committee's recommendation with the FDA and/or the EMA and the COBALT trial cannot be timely completed, cannot be completed as designed or fails, we may not be able to maintain our previously granted marketing approval of Ocaliva for PBC. Similarly, if approved based on a surrogate endpoint, continued approval of OCA for other indications, or of any of our other product candidates, may be contingent upon the verification and description of clinical benefit in confirmatory trials.

Our marketing authorization in the European Union for Ocaliva for the treatment of PBC is not a full approval. Instead, it is conditional on the conduct of certain post-approval studies, including the COBALT trial. Our ability to maintain conditional marketing authorization of Ocaliva for PBC in the European Union is limited to specific circumstances and subject to several conditions and obligations that we may be unable to satisfy in whole or at all, including the completion of one or more clinical outcomes trials to confirm the clinical benefit of Ocaliva for PBC. Conditional marketing authorizations based on incomplete clinical data may be granted for a limited number of listed medicinal products for human use, including products designated as orphan medicinal products under European Union law, if (i) the risk-benefit balance of the product is positive, (ii) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (iii) unmet medical needs will be fulfilled and (iv) the benefit to public health of the immediate availability on the market of the medicinal product outweighs the risk inherent in the fact that additional data are still required. Specific obligations, including obligations relating to the timely and successful completion of ongoing or new studies and the collection of pharmacovigilance data, may be specified in the conditional marketing authorization. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions. Although we have successfully renewed our conditional marketing authorization in the European Union in the past, there can be no assurance that we will be able to continue to do so in the future. Failure to renew our conditional marketing authorization would prevent us from continuing to market Ocaliva for PBC in Europe.

Our ongoing Phase 3 REGENERATE trial of OCA in patients with liver fibrosis due to NASH incorporates an interim primary surrogate endpoint that may serve as the basis for accelerated approval in the United States and as the basis for a conditional approval in Europe. Accelerated approval in the United States and conditional approval in the European Union for OCA for liver fibrosis due to NASH are subject to similar risks as discussed above in relation to OCA for PBC. In the REGENERATE primary efficacy analysis, once-daily OCA 25 mg met, with statistical significance, the primary endpoint agreed with the FDA of fibrosis improvement by at least one stage with no worsening of NASH (defined as no worsening of hepatocellular ballooning, no worsening of lobular inflammation and no worsening of steatosis) at the planned 18-month analysis. Although a numerically greater proportion of patients in both OCA treatment groups compared to placebo achieved the primary endpoint of NASH resolution with no worsening of liver fibrosis in the primary efficacy analysis, this result did not reach statistical significance. As agreed with the FDA, in order for the primary objective to be met, the study was required to achieve one of the two primary endpoints. Notwithstanding the results of the REGENERATE 18-month analysis, the recent CRL issued by the FDA indicated that, based on the data the FDA had reviewed, the FDA had determined that the predicted benefit of OCA based on a surrogate histopathologic endpoint remains uncertain and does

not sufficiently outweigh the potential risks to support accelerated approval for the treatment of patients with liver fibrosis due to NASH. The FDA recommended that we submit additional post-interim analysis efficacy and safety data from the ongoing REGENERATE study in support of potential accelerated approval and that the long-term outcomes phase of the study should continue. Although we are in discussions with the FDA with respect to the potential resubmission of our NDA seeking accelerated approval of OCA for the treatment of liver fibrosis due to NASH, there is no assurance that we will be successful or that OCA will be approved for liver fibrosis due to NASH on an accelerated basis, or at all. Furthermore, in November 2018, the EMA issued a draft position paper in which it presented its preliminary views with respect to various NASH clinical development matters, including with respect to potential surrogate endpoints, and requested comments thereon by August 2019. Although we did not reach agreement with the EMA on the definition and analysis of a surrogate endpoint prior to the readout of the 18-month analysis of the REGENERATE trial, we believe that the totality of the REGENERATE interim analysis data supports the MAA we filed with the EMA. However, the data that we have submitted to the EMA may not ultimately be found by the EMA to be sufficient for marketing approval on a conditional basis, or at all. In June 2019, the FDA issued new draft guidance on the development of drugs for the treatment of NASH patients with compensated cirrhosis. Although we believe that, if successful, our Phase 3 REVERSE trial will support a regulatory submission seeking accelerated approval of OCA for liver fibrosis due to NASH with compensated cirrhosis in the U.S., we do not know if achievement of the primary endpoint will ultimately be found sufficient by the FDA for approval on an accelerated basis, or at all.

While OCA received breakthrough therapy designation from the FDA in January 2015 for the treatment of NASH patients with liver fibrosis and we have filed a NDA in the United States and a MAA in Europe for approval of OCA for liver fibrosis due to NASH based on the results from the 18-month interim analysis of our Phase 3 REGENERATE trial, we do not know if one pivotal clinical trial will be sufficient for marketing approval or if regulatory authorities in the United States, Europe or our other target markets will approve OCA for liver fibrosis due to NASH on an accelerated or conditional basis, or at all. There may be delays in the FDA and EMA review processes and the FDA and/or the EMA may also require that we continue our Phase 3 REGENERATE trial until completion to assess the potential benefits of OCA treatment on liver-related and other clinical outcomes. Our regulatory pathway for OCA for the treatment of NASH will depend upon our discussions with the FDA and the EMA. As a result, we may face difficulty in establishing an acceptable registration strategy with respect to our Phase 3 REGENERATE and REVERSE trials, as well as other trials we may conduct in other subpopulations of NASH patients.

Prior to any approval of OCA for liver fibrosis due to NASH or OCA for PBC in jurisdictions in which it is not currently approved or the approval of our other product candidates, the FDA, EMA or other applicable regulatory authorities may require additional preclinical studies and/or clinical trials, which may be expensive and time consuming to conduct and complete. Consequently, any such requirement that we conduct additional preclinical studies or clinical trials could materially and adversely affect our business, financial condition and results of operations. Furthermore, even if we receive such approval, any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of our approved products could impact our commercial success in our target markets.

***Delays or difficulties in the commencement, enrollment and completion of our clinical trials and studies could increase our product development costs and delay, limit or prevent us from obtaining regulatory approval for OCA and our other product candidates.***

Delays or difficulties in the commencement, enrollment and completion of our clinical trials and studies could increase our product development costs and limit or prevent us from obtaining or maintaining regulatory approval for OCA and our other product candidates. We are currently conducting a number of clinical trials, including our Phase 4 COBALT clinical outcomes confirmatory trial of Ocaliva for PBC, our Phase 3 REGENERATE trial of OCA in patients with liver fibrosis due to NASH through clinical outcomes in order to confirm clinical benefit and our Phase 3 REVERSE trial of OCA for NASH patients with compensated cirrhosis. We are also conducting our CARE trial of OCA in pediatric patients with biliary atresia as a part of an EMA-approved PIP supporting the conditional approval of Ocaliva for PBC. The results from these clinical trials and our other clinical trials and studies may not be available when we anticipate and we may be required to conduct additional clinical trials or studies not currently planned in order for our product candidates, including OCA for PBC and NASH, to be approved or to maintain approvals in the U.S., Europe or the other jurisdictions in which our products are approved. In addition, our clinical programs are subject to a number of risks and uncertainties, such as the results of other trials, patient enrollment, safety issues or regulatory interactions that could result in a change of trial

design or timing. For example, due to COVID-19 we paused screening and randomization in our Phase 4 COBALT confirmatory outcomes trial evaluating Ocaliva for PBC and in our Phase 2 study evaluating bezafibrate in combination with OCA for PBC and have taken measures intended to minimize disruptions and protect and retain enrolled patients participating in such trials. While we recommenced enrollment in our COBALT trial and in our Phase 2 study evaluating bezafibrate in combination with OCA, any delays or difficulties completing such trials as a result of the pause in enrollment could increase our product development costs and limit or prevent us from obtaining or maintaining regulatory approval. Consequently, we do not know whether our current or future clinical trials or studies of OCA or our other product candidates will be completed on schedule, if at all.

The commencement, enrollment and completion of our clinical trials and studies may be delayed, suspended or otherwise adversely affected for a variety of reasons, including:

- our inability to obtain sufficient funds to complete or continue our clinical trials;
- our inability to reach agreements on acceptable terms with prospective contract research organizations (“CROs”) and trial sites, the terms of which may be subject to extensive negotiation and may vary significantly among our various CROs and trial sites;
- clinical holds, other regulatory objections to our commencing or continuing a clinical trial or our inability to obtain regulatory approval to commence clinical trials in countries that require such approvals;
- our discussions with the FDA, EMA or other regulatory authorities prior to, or following, the initiation of our clinical trials, regarding, among other matters, the scope or design of our clinical trials, including trial endpoints, protocols and statistical analysis plans, and any modifications thereto;
- our inability to identify and maintain a sufficient number of trial sites, many of which may already be engaged in other clinical trial programs, including some that may be for the same indications targeted by our product candidates;
- any delay in receiving results from, or failure to achieve the necessary results in, our clinical trials;
- our inability to obtain approval from institutional review boards or independent ethics committees to conduct our clinical trials at their respective sites;
- any data monitoring committee recommendation that our clinical trials be modified, suspended or terminated due to safety, lack of efficacy or other reasons;
- severe or unexpected drug-related adverse events experienced by patients or any determination that a clinical trial presents unacceptable health risks;
- any breach of the terms of any relevant agreement by us, our current or future collaborators that have responsibility for the clinical development of any of our product candidates or investigators conducting clinical trials on our product candidates;
- our inability to timely manufacture, or obtain from our contract manufacturers, sufficient quantities of our product candidate required for our clinical trials; and
- any difficulty recruiting, enrolling or retaining patients in our clinical trials based on, among other factors, the enrollment criteria for our clinical trials, the rarity of the disease, the characteristics of the population being studied, the risks of the procedures that may be required as part of the clinical trials, such as a liver biopsy, the availability of our products to patients generally following the approval of such products or competition from other clinical trial programs recruiting patients for the same indications as our product candidates.

For example, our Phase 3 REGENERATE trial is a large and complicated clinical trial in a disease without any approved therapies and involves serial liver biopsies over many years. While we announced topline results from the 18-month analysis of our pivotal Phase 3 REGENERATE trial in February 2019, the study is currently planned to continue through clinical outcomes in order to confirm clinical benefit and there can be no assurance that we will retain a sufficient number of patients in the full study cohort or complete the clinical outcomes trial in accordance with the study protocol or on a timely basis, if at all. Similarly, our COBALT clinical outcomes confirmatory trial for PBC includes subjects across the spectrum of PBC disease, including early and advanced PBC. Enrolling and retaining patients in such trials is challenging and there can be no assurance that we will enroll and retain a sufficient number of patients in the full study or complete the clinical outcomes trial in accordance with the study protocol or on a timely basis, if at all. As we engage in other large and complicated trials and trials in advanced disease populations, we may experience a number of challenges that may negatively affect or delay our plans and development programs.

We have in the past experienced difficulties enrolling and retaining patients enrolled in our clinical trials. Difficulties in enrolling and retaining patients, including due to COVID-19, may delay our clinical trials or result in negative or inconclusive outcomes, and we or our collaborators may decide, or regulatory authorities may require us, to conduct additional clinical trials or additional analyses of existing clinical trials. Any delay or compromises with respect to the validity of our clinical trials may have a material adverse effect on our business or decrease our competitive position relative to other biotechnology or pharmaceutical companies with whom we compete.

In addition, if we or any of our collaborators are required to conduct additional preclinical or clinical studies or other development work on our product candidates beyond that contemplated, our ability to obtain regulatory approval of these product candidates and generate revenue from their sales would be similarly harmed.

***COVID-19 could materially and adversely affect our clinical trials.***

COVID-19 has spread to the U.S., Europe, and other countries in which we are engaged in, or plan to engage in, clinical development activities. We are closely monitoring the COVID-19 outbreak, have temporarily paused screening and randomization in our ongoing PBC trials during periods of 2020 and, together with our contract research organizations, study sites and other partners, have taken measures intended to minimize disruptions and protect and retain patients enrolled in our clinical trials, including, where appropriate, the use of telemedicine, home care visits, direct delivery of investigational product and other measures. Notwithstanding our efforts, some of the sites participating in our clinical trials have been affected by site closings or reduced capacity, particularly in regions that are experiencing heightened impact from COVID-19. While we are monitoring the situation closely, if there was a meaningful negative impact on the data capture or data quality of any of our clinical trials, such trials may not be successful or we could be required to repeat, extend the duration of, increase the size of, or otherwise modify such trials, which could prevent or significantly delay the potential commercialization of our product candidates and require greater expenditures. We cannot at this time predict with certainty the scope of the impact of COVID-19 on our ability to execute our clinical trials. The extent to which COVID-19 may impact our clinical trials will depend on future developments, which are highly uncertain, such as the geographic spread of the disease, the duration of the outbreak, public health restrictions on travel and in-person interactions, business closures and disruptions, and the effectiveness of actions taken to contain and treat the disease. As a result, our clinical trials may not be successful or we may experience issues due to COVID-19 that could severely impact our clinical trials, including:

- delays, interruptions or difficulties in the enrollment, scheduling and retention of patients in our clinical trials;
- delays, interruptions or difficulties in the conduct of key clinical trial activities, such as clinical trial site monitoring and inspection readiness activities;

## [Table of Contents](#)

- trial conduct issues, including protocol deviations (e.g., failure to timely collect liver biopsies or other required laboratory data), data capture issues and data quality issues;
- delays or interruptions in the supply or administration of investigational product to patients in our clinical trials;
- delays or interruptions in the supply of necessary equipment or materials to clinical sites;
- delays or difficulties obtaining approvals from regulatory authorities, institutional review boards or ethics committees of clinical trial protocols and related clinical documentation (or amendments and addendums thereto);
- delays, interruptions or difficulties in clinical site initiations, including in connection with the recruitment of clinical site investigators and clinical site staff;
- the redeployment of healthcare resources, including clinical site investigators and clinical site staff supporting the conduct of our clinical trials, to assist in the treatment of COVID-19 patients;
- the diversion of human capital, including employees, independent contractors, vendors and other third parties, otherwise focused on the conduct of our clinical trials due to sickness, safety concerns or government or employer imposed travel or working restrictions;
- new federal, state and local government regulations or guidance that require us to change the way we conduct our clinical trials, require the interruption or termination of our clinical trials or that result in significant and unexpected new costs;
- delays or difficulties in interactions with regulatory authorities, institutional review boards, ethics committees and key consultants and vendors due to layoffs, temporary leaves, terminations or other actions limiting available employee resources; and
- the refusal of regulatory authorities to accept clinical trial data from clinical trials that have been negatively affected by COVID-19.

Any such delay, interruption or issue could materially and adversely affect our business, financial condition and results of operations.

***Failure can occur at any stage of clinical development. The results of earlier clinical trials are not necessarily predictive of future results and any product candidate we or our collaborators advance through clinical trials, including OCA, may not have favorable results in later clinical trials or receive or maintain regulatory approval.***

Clinical failure can occur at any stage of clinical development. Clinical trials may produce negative or inconclusive results, and we or our collaborators may decide, or regulators may require us, to conduct additional clinical trials or preclinical studies. In addition, data obtained from trials and studies are susceptible to varying interpretations, and regulators may not interpret our data as favorably as we do, which may delay, limit or prevent regulatory approval. Success in preclinical studies and early clinical trials does not ensure that subsequent clinical trials will generate the same or similar results or otherwise provide adequate data to demonstrate the efficacy and safety of our product candidates. A number of companies in the pharmaceutical industry, including those with greater resources and experience than us, have suffered significant setbacks in Phase 3 clinical trials and at other stages of clinical development, even after seeing promising results in earlier clinical trials.

In addition, the design of clinical trials, including trial endpoints, protocols and statistical analysis plans, can determine whether such trials will support product approvals, and flaws in the design of such trials may not become apparent until such trials are well-advanced. We may be unable to design and execute clinical trials to support regulatory approval.

Further, clinical trials of product candidates often reveal that it is not practical or feasible to continue development efforts. If OCA or our other product candidates are found to be unsafe or lack sufficient efficacy for any indication, we will not be able to obtain or maintain regulatory approval for them, and our prospects and business may be materially and adversely affected.

There may be significant variability in the safety and/or efficacy results we see in different trials studying OCA or our other product candidates due to numerous factors, including differences in the underlying disease being studied, changes or differences in trial protocols or statistical analysis plans, differences in the composition of the patient populations or clinical trial sites, differences in adherence to the dosing regimen and other aspects of the trial protocols and differences in the rate of dropouts among clinical trial participants. We do not know whether any Phase 2, Phase 3 or other clinical trials we or any of our collaborators may conduct on our product candidates will demonstrate consistent or adequate efficacy and safety or result in the approval of our product candidates by regulatory authorities. If we are unable to bring any of our current or future product candidates to market, acquire any previously approved products or maintain approval for our approved products, our ability to create long-term stockholder value will be limited.

Although Ocaliva for PBC has received accelerated approval in the United States and conditional approval in the European Union, its full approval depends on the timely completion and results of post-marketing clinical trials, including our Phase 4 COBALT trial. We cannot assure you that these trials will be timely completed or that the results of such trials will support the full approval of Ocaliva in the United States, European Union or our other target markets where Ocaliva has not received full approval.

In December 2014, we received comprehensive datasets from the Phase 2b FLINT trial for the treatment of NASH, which met its primary endpoint with statistical significance. In October 2015, we announced that the Phase 2 dose ranging trial of OCA in 200 adult NASH patients in Japan conducted by our former collaborator, Sumitomo Dainippon, did not meet its primary endpoint with statistical significance. In the Sumitomo Dainippon trial, there was a dose dependent, although not statistically significant, increase in the percentage of OCA-treated patients compared to placebo who achieved the primary endpoint ( $p = 0.053$ ). In addition, no difference was seen in fibrosis improvement in the OCA groups compared to placebo. The Sumitomo Dainippon Phase 2 trial involved different doses of OCA being administered to the trial subjects than those utilized in the Phase 2b FLINT trial. Furthermore, the baseline characteristics between the patients in the Japanese Phase 2 dose ranging trial conducted by Sumitomo Dainippon were distinct in a number of ways from those of the Western patients included in the Phase 2b FLINT trial.

In February 2019, we announced topline results from the 18-month analysis of our pivotal Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH. In the primary efficacy analysis, once-daily OCA 25 mg met, with statistical significance, the primary endpoint agreed with the FDA of fibrosis improvement by at least one stage with no worsening of NASH (defined as no worsening of hepatocellular ballooning, no worsening of lobular inflammation and no worsening of steatosis) at the planned 18-month analysis. Although a numerically greater proportion of patients in both OCA treatment groups compared to placebo achieved the primary endpoint of NASH resolution with no worsening of liver fibrosis in the primary efficacy analysis, this result did not reach statistical significance. As agreed with the FDA, in order for the primary objective to be met, the study was required to achieve one of the two primary endpoints. Notwithstanding the results of the REGENERATE 18-month analysis, the recent CRL issued by the FDA indicated that, based on the data the FDA had reviewed, the FDA had determined that the predicted benefit of OCA based on a surrogate histopathologic endpoint remains uncertain and does not sufficiently outweigh the potential risks to support accelerated approval for the treatment of patients with liver fibrosis due to NASH. The FDA recommended that we submit additional post-interim analysis efficacy and safety data from the ongoing REGENERATE study in support of potential accelerated approval and that the long-term outcomes phase of the study should continue. Although we are in discussions with the FDA with respect to the potential resubmission of our NDA seeking accelerated approval of OCA for the treatment of liver fibrosis due to NASH, there is no assurance that we will be successful or that OCA will be approved for liver fibrosis due to NASH on an accelerated basis, or at all. While OCA received breakthrough therapy designation from the FDA in January 2015 for the treatment of NASH patients with liver fibrosis and we filed a NDA in the United States and a MAA in Europe for approval of OCA for liver fibrosis due to NASH based on the results from the 18-month analysis of our Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH, we do not know if one pivotal clinical trial will be sufficient for marketing approval or if regulatory authorities in the United States, Europe or our other target markets will approve OCA for liver fibrosis due to NASH on an accelerated or conditional basis, or at all. Additionally, interim

analysis results at 18 months were based on surrogate endpoints and the impact on clinical outcomes has not been confirmed. Our Phase 3 REGENERATE trial remains ongoing through clinical outcomes for verification and description of clinical benefit of OCA for liver fibrosis due to NASH.

***Our product candidates may have undesirable side effects which may delay or prevent marketing approval, or, if approval is received, require that our products be taken off the market or include new or additional safety warnings. Any such events may limit our existing and future product sales and materially and adversely affect our business, financial condition and results of operations.***

OCA has been shown to be a potent FXR agonist. With the exception of the endogenous human bile acid chenodeoxycholic acid and cholic acid, there are no approved FXR agonists and the adverse effects from long-term exposure to this drug class are unknown. Unforeseen side effects from any of our product candidates, including OCA, could arise either during clinical development or, if approved, after the approved product has been marketed. Serious adverse events, including deaths, in patients taking OCA have occurred in clinical trials and in the post-marketing setting, and we cannot assure you that additional serious adverse events in patients taking OCA in clinical trials or in the post-marketing setting will not occur.

The most common side effects observed in clinical trials of OCA for PBC were pruritus, fatigue, headaches, nausea, constipation and diarrhea. In our Phase 3 POISE trial, pruritus, generally mild to moderate, was the most frequently reported adverse event associated with OCA treatment for PBC and was observed in 38% of patients on placebo, 70% of patients in the OCA 10 mg group and 56% of patients in the OCA titration group (5 mg to 10 mg). Eight patients discontinued due to pruritus, of whom none were in the placebo group, seven (10%) were in the OCA 10 mg group and one (1%) was in the OCA titration group. Pruritus also has been observed in other clinical trials of OCA. Decreases in high density lipoprotein HDL cholesterol were also observed during treatment in our Phase 3 POISE trial. In our Phase 2 trials for OCA for PBC, a dose-response relationship was observed in the occurrence of liver-related adverse reactions, including jaundice, ascites and primary biliary cholangitis flare with dosages of OCA of 10 mg once daily to 50 mg once daily (up to 5-times the highest recommended dosage), as early as one month after starting treatment with OCA. The European label for Ocaliva also notes that elevations in alanine amino transferase and aspartate aminotransferase were observed in patients treated with OCA.

In the course of our post-marketing pharmacovigilance activities, deaths have been reported in PBC patients with moderate or severe hepatic impairment. In an analysis performed by us and in consultation with the FDA, we concluded that certain of these patients were prescribed once daily doses of Ocaliva, which is seven times higher than the recommended weekly dose in such patients. As a result, in September 2017, we issued a Dear Health Care Provider (“DHCP”) letter, and the FDA also subsequently issued its own drug safety communication to reinforce recommended label dosing. Both communications remind healthcare providers of the importance of the recommended reduced dosing of Ocaliva in PBC patients with moderate or severe hepatic impairment, while reiterating the importance of monitoring PBC patients for progression of their disease and the occurrence of liver-related adverse reactions. In addition to the DHCP letter, we took actions to enhance education about appropriate use of Ocaliva. These initiatives included: reeducating physicians on the label, with a focus on ensuring appropriate dosing for patients with moderate or severe hepatic impairment; enhancing monitoring of patients for liver-related adverse reactions; and adjudicating reported cases of serious liver injury, including in patients with no or mild hepatic impairment. In February 2018, we announced that the Ocaliva label in the United States had been updated by the FDA to include a boxed warning and a dosing table that reinforced the then-existing dosing schedule for patients with Child-Pugh Class B or C or decompensated cirrhosis. In addition, the FDA issued an updated drug safety communication to accompany the revised label. We remain focused on the safety of all of the patients using Ocaliva within and outside of our ongoing clinical studies and have engaged with relevant regulatory authorities to ensure that the Ocaliva label sufficiently reinforces the importance of appropriate dosing in patients with advanced cirrhosis.

The FDA has notified us that, in the course of its routine safety surveillance, in May 2020 the FDA began to evaluate a newly identified safety signal, or NISS, regarding liver disorder for Ocaliva which the FDA classified as a potential risk. Pursuant to the FDA’s guidance, this does not mean that the FDA has concluded that the drug has the listed risk or that the FDA has identified a causal relationship between Ocaliva and the potential risk. The FDA has informed us that its review of the NISS is focused on a subset of the cirrhotic, or more advanced, PBC patients who have taken Ocaliva. As part of

our routine pharmacovigilance efforts, we have worked with the FDA to reconcile our internal safety database with the FDA Adverse Event Reporting System database and are now conducting a comprehensive assessment of all available data, including data from our completed clinical trials, blinded reviews of ongoing clinical trial data, unblinded reviews of ongoing clinical trial data by the data monitoring committee, post-marketing data and natural history data. Any safety concerns associated with Ocaliva, perceived or real, may adversely affect the successful development and commercialization of our product candidates and approved products, including Ocaliva, and materially and adversely affect our business.

Ocaliva is contraindicated for PBC patients with complete biliary obstruction in the United States and the European Union. For PBC patients with HDL reductions and no response to Ocaliva after one year at the maximum tolerated dose, the U.S. label asks prescribing physicians to weigh the risks against the benefits of continuing treatment.

In the 18-month analysis of our pivotal Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH, the safety population included 1,968 randomized patients who received at least one dose of investigational product (OCA or placebo) with exposures up to 37 months. Adverse events were generally mild to moderate in severity and the most common were consistent with the known profile of OCA. The frequency of serious adverse events was similar across treatment groups (11% in placebo, 11% in OCA 10 mg and 14% in OCA 25 mg), and no serious adverse event occurred in > 1% of patients in any treatment group. There were 3 deaths in the study (2 in placebo: bone cancer and cardiac arrest and 1 in OCA 25 mg: glioblastoma) and none were considered related to treatment. The most common adverse event reported was dose-related pruritus (19% in placebo, 28% in OCA 10 mg and 51% in OCA 25 mg). The incidence of pruritus across all three treatment groups was highest in the first three months and decreased thereafter. The large majority of pruritus events were mild to moderate, with severe pruritus occurring in a small number of patients (< 1% in placebo, < 1% in OCA 10 mg and 5% in OCA 25 mg). A higher incidence of pruritus-associated treatment discontinuation was observed for OCA 25 mg (< 1% in placebo, < 1% in OCA 10 mg and 9% in OCA 25 mg). According to the clinical study protocol, investigator assessed severe pruritus mandated treatment discontinuation. Consistent with observations from previous NASH studies, OCA treatment was associated with an increase in low density lipoprotein (“LDL”) cholesterol, with a peak increase of 22.6 mg/dL at 4 weeks and subsequently reversing and approaching baseline at month 18 (4.0 mg/dL increase from baseline). Triglycerides rapidly and continually decreased in the OCA treatment groups through month 18. There were few and varied serious cardiovascular events and incidence was balanced across the three treatment groups (2% in placebo, 1% in OCA 10 mg and 2% in OCA 25 mg). In patients with type 2 diabetes, OCA treatment was associated with an early transient increase in fasting glucose and hemoglobin A1c with return to levels similar to placebo by month 6. No clinically meaningful changes were noted in non-diabetic patients.

With respect to hepatobiliary events, more patients (3%) on OCA 25 mg experienced gallstones or cholecystitis compared to < 1% on placebo and 1% on OCA 10 mg. While hepatic serious adverse events were rare (< 1% incidence in each of the three treatment groups), more occurred in the OCA 25 mg group with no pattern attributable to OCA.

In the Phase 2b FLINT trial, pruritus occurred more frequently in the OCA treatment group than in the placebo treatment group (23% vs. 6%,  $p < 0.0001$ ) and at a higher grade (predominately moderate pruritus). OCA treatment was also associated with changes in serum lipid levels, including increases in total cholesterol and LDL cholesterol and a decrease in HDL cholesterol, that were observed within 12 weeks of initiating treatment, peaked and then decreased in magnitude while on treatment, and reversed further during the 24-week post-treatment period. These changes in cholesterol levels, along with the achievement of pre-defined efficacy criteria, played a role in the decision of the FLINT data and safety monitoring board to terminate the treatment phase of the Phase 2b FLINT trial, and the publication of the FLINT results noted the need for further study of these changes. There were two patient deaths in the Phase 2b FLINT trial, and neither death was considered related to OCA treatment.

Furthermore, the Phase 2 dose ranging trial of OCA in 200 adult NASH patients in Japan conducted by our former collaborator, Sumitomo Dainippon, did not meet statistical significance for the primary endpoint. The primary endpoint in the Sumitomo Dainippon trial was histologic improvement defined as at least a two-point improvement in the nonalcoholic fatty liver disease activity score with no worsening of fibrosis. In this trial, there was a dose dependent, although not statistically significant, increase in the percentage of OCA-treated patients compared to placebo who achieved the primary endpoint ( $p = 0.053$ ). In addition, no difference was seen in fibrosis improvement in the OCA groups compared to placebo.

In December 2015, we initiated a Phase 2 clinical trial, known as the CONTROL trial, to characterize the lipid metabolic effects of OCA and cholesterol management effects of concomitant statin administration in NASH patients. CONTROL enrolled 80 NASH patients who were naïve to statin therapy or had undergone a statin washout period. The study included a 16-week double-blind phase followed by an optional long-term safety extension (“LTSE”) phase of the trial. OCA treatment in the absence of statin therapy over the first four weeks resulted in an increase in LDL across all OCA treatment groups, while the placebo group was relatively unchanged. Treatment with atorvastatin beginning at week four and continuing through week 16 reversed OCA-related increases in LDL to below baseline levels in all OCA treatment groups. Dose-dependent pruritus was the most common adverse event in patients treated with OCA, occurring in 5% of patients on placebo, 5% of patients in the OCA 5 mg group, 10% of patients in the OCA 10 mg group and 55% of patients in the OCA 25 mg group. All adverse events were mild to moderate and two patients discontinued treatment in the OCA 25 mg group due to pruritus. Over 95% of the patients completing the double-blind phase of CONTROL enrolled in the LTSE phase of the trial.

During the LTSE phase of CONTROL, there was one patient death. This patient was a 64 year-old male with a history of NASH associated liver cirrhosis, morbid obesity (BMI >40) and type 2 diabetes. At baseline, this patient had blood tests consistent with impaired liver function (e.g., low LDL and low platelets). The patient was randomized to placebo for the double-blind phase of the study. Early in the double-blind phase, the patient had serum biochemistry changes consistent with worsening hepatic impairment (e.g., albumin decline and bilirubin was increasing). Atorvastatin was started per protocol and then stopped early due to the patient’s persistently low LDL levels. The patient later enrolled in the LTSE phase and began receiving OCA 25 mg treatment. Over the following four months, the patient’s serum biochemistry remained consistent with ongoing hepatic impairment. Approximately five months after starting the LTSE phase, the patient developed severe protracted diarrhea, which resulted in weight loss of 30 pounds over the ensuing one-month period. Both an infectious cause and possible inflammatory bowel disease were suspected, and the patient subsequently was started on broad spectrum antibiotics and steroid therapy. Due to the diarrhea, the principal investigator stopped treatment with OCA and discontinued the patient from the study. Concurrently, the patient reported jaundice and was found to have significantly elevated serum bilirubin and ALP, while other liver enzymes remained relatively stable. Over the ensuing two-week period, various diagnostic tests and procedures were performed (e.g., magnetic resonance cholangiopancreatography to investigate possible gallstone bile duct obstruction) and the patient continued receiving a number of other medications, including the ongoing course of steroid therapy. During this time, the patient continued to deteriorate and was hospitalized with acute renal and liver failure, complicated by severe metabolic acidosis. The patient rapidly progressed to multi-organ system failure, sepsis and death. The principal investigator determined that the events leading to the patient’s death were unlikely related to OCA. Despite the numerous confounding factors in this case, given the contemporaneous administration of OCA during the patient’s ongoing deterioration, we determined that it could not be ruled out that these events were possibly related to treatment. Subsequent to our determination, the independent data safety monitoring committee separately evaluated the case and determined that the events leading to the patient’s death were unlikely related to OCA.

Additional or unforeseen side effects relating to OCA or any of our other product candidates could arise either during clinical development or, if approved, after the approved product has been marketed. With the approval of Ocaliva for PBC in the United States, Europe and certain of our other target markets, OCA is currently used in an environment that is less rigorously controlled than in clinical studies. If new side effects are found, if known side effects are shown to be more severe than previously observed or if OCA is shown to have other unexpected characteristics, we may need to abandon our development of OCA for PBC, NASH and other potential indications. Furthermore, our commercial sales of Ocaliva for PBC may be materially and adversely affected.

The range and potential severity of possible side effects from systemic therapies is significant. The results of our current or future clinical trials may show that our product candidates, including OCA, cause undesirable or unacceptable side effects, which could interrupt, delay or halt clinical trials, result in a delay of, or failure to obtain, marketing approval from the FDA and other regulatory authorities, result in marketing approval from the FDA and other regulatory authorities with restrictive label warnings or result in the withdrawal of previously granted marketing approvals.

In addition, our product candidates are being developed as potential treatments for severe, life threatening diseases and, as a result, our trials will necessarily be conducted in patient populations that are more prone than the general population to exhibit certain disease states or adverse events. For example, our Phase 3 REVERSE trial in NASH patients

with compensated cirrhosis has expanded our NASH development program into a more advanced NASH patient population and accordingly imposes certain eligibility requirements for up-titration, as well as certain monitoring requirements thereafter. Ocaliva is prescribed in patients suffering from various stages of PBC, which can be life threatening, and patients may suffer from other concomitant illnesses that may increase the likelihood of certain adverse events. It may be difficult to discern whether certain events or symptoms observed during our clinical trials or by patients using our approved products are related to our product candidates or approved products or some other factor. As a result, we and our development programs may be negatively affected even if such events or symptoms are ultimately determined to be unlikely related to our product candidates or approved products. We cannot assure you that additional or more severe adverse side effects related to OCA or our other product candidates will not be observed in our clinical trials or in the commercial setting. If observed, such adverse side effects could delay or preclude regulatory approval of OCA, limit commercial use or result in the withdrawal of previously granted marketing approvals.

If we or others identify undesirable or unacceptable side effects caused by our product candidates or products:

- we may be required to modify, suspend or terminate our clinical trials;
- we may be required to modify or include additional dosage and administration instructions, warnings and precautions, contraindications, boxed warnings, limitations, restrictions or other statements in the product label for our approved products, or issue field alerts to physicians and pharmacies or implement other risk mitigation programs;
- we may be required to conduct costly additional clinical trials;
- we may be subject to limitations on how we may promote our approved products;
- sales of our approved products may decrease significantly;
- regulatory authorities may require us to take our approved products off the market;
- we may be subject to regulatory investigations, government enforcement actions, litigation or product liability claims; and
- our products may become less competitive or our reputation may suffer.

***Breakthrough therapy designation for OCA may not lead to faster development or regulatory processes or increase the likelihood that the FDA will approve OCA for the treatment of NASH patients with fibrosis.***

If a drug is intended for the treatment of a serious or life-threatening condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development, the FDA may grant a breakthrough therapy designation. Breakthrough therapy designation is intended to facilitate the development, and expedite the review, of such drugs, but the breakthrough therapy designation does not assure marketing approval by the FDA.

In January 2015, we received breakthrough therapy designation for OCA for the treatment of NASH patients with liver fibrosis. However, there is no guarantee that the receipt of breakthrough therapy designation will result in a faster development process, review or approval of OCA for liver fibrosis due to NASH or increase the likelihood that OCA will be granted marketing approval for NASH patients with liver fibrosis. Notwithstanding our receipt of breakthrough therapy designation, in June 2020 we received a CRL from the FDA with respect to our NDA for OCA for liver fibrosis due to NASH. Although we are in discussions with the FDA with respect to the potential resubmission of our NDA seeking accelerated approval of OCA for the treatment of liver fibrosis due to NASH, there is no assurance that the outcome of these discussions will lead to resolution of the issues identified in the CRL or that if we resubmit our NDA that it will be approved. Similarly, any future breakthrough therapy designation relating to any other potential indication of OCA or our

other product candidates will neither guarantee a faster development process, review or approval nor improve the likelihood of the grant of marketing approval by the FDA compared to conventional FDA procedures. In addition, the FDA may withdraw any breakthrough therapy designation at any time. While we may seek breakthrough therapy designation for one or more of our product candidates in the future, we can give no assurance that the FDA will grant such status.

***We may not be able to obtain or, if approved, maintain orphan drug exclusivity for our approved products or product candidates, which could cause our revenues to suffer.***

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs and biologics for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. OCA has received orphan drug designation in the United States and the European Union for the treatment of PBC and PSC.

Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same product during the exclusivity period. The applicable exclusivity period is seven years in the United States and ten years in Europe. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure a sufficient quantity of the product to meet the needs of patients with the rare disease or condition. In addition, the European exclusivity period can be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation because, for example, the product is sufficiently profitable not to justify maintenance of market exclusivity.

Any failure to maintain orphan drug status may subject us to mandatory price discounts in Europe and result in the loss of other benefits, such as tax exemptions for sales. As such, the loss of orphan drug status may have a negative effect on our ability to successfully commercialize our products, earn revenues and achieve profitability.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. Even after an orphan drug is approved, the FDA or EMA may subsequently approve another product for the same condition if the FDA or EMA concludes that the later product is clinically superior (i.e., it is shown to be safer, more effective or makes a major contribution to patient care). Any inability to secure or maintain orphan drug status or the exclusivity benefits of this status could have a material adverse impact on our ability to develop and commercialize our product candidates and approved products.

***We rely entirely on third parties for the manufacture of our product requirements for our preclinical studies and clinical trials, as well as our commercial supply of Ocaliva and, if approved, OCA for liver fibrosis due to NASH and our other product candidates, and also depend on third-party vendors and CROs for certain of our clinical trial and product development activities. Our business could be harmed if our third-party manufacturers fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices, or if our third-party vendors or CROs assisting us with our clinical trials and product development activities fail to comply with their contractual commitments or applicable regulatory obligations or if we lose our relationships with our third-party vendors and CROs.***

We do not manufacture the pharmaceutical products that we sell or the product candidates that we are developing. We rely on third-party contract manufacturers for all of our required raw materials, active pharmaceutical ingredient and finished product for our commercial sales and for our existing and anticipated clinical trials and preclinical studies. Any inability by our contract manufacturers to continue to provide services to us for any reason, including due to COVID-19 and related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions, business closures and disruptions and other public health safety measures, could disrupt the supply chain for our pharmaceutical products and product candidates and materially and adversely affect our commercialization efforts and clinical development program, and we may be unable to identify, qualify and engage replacement suppliers on terms that are favorable to us on a timely basis, if at all. The extent to which COVID-19 impacts the operations of our third-party contract manufacturers will depend on future developments, which are highly uncertain and cannot be predicted with confidence,

including the geographic spread of the disease, the duration of the outbreak, public health safety measures and the effectiveness of the actions taken to contain and treat the disease.

We currently have an agreement with PharmaZell GmbH for the manufacture and commercial supply of API for use in Ocaliva and, if approved, OCA for liver fibrosis due to NASH, but expect to shift our longer-term API supply requirements to other suppliers. While we have procured supplies of API for the commercialization of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH that we believe will be sufficient to meet our requirements during the initial stages of a potential NASH launch, we may not be able to procure sufficient supplies of API on an ongoing basis. To address this concern, we have qualified an additional API supplier from which we may currently acquire API on a purchase order basis and continue to engage in activities intended to ensure that our long-term commercial supply requirements are satisfied. Despite these efforts, we may not be able to meet our long-term commercial supply requirements of API for the manufacture of Ocaliva or, if approved, OCA for liver fibrosis due to NASH or other indications on acceptable terms, or at all. We do not have agreements for long-term supplies of any of our product candidates other than OCA. We currently obtain supplies and services relating to our other product candidates from our third-party contract manufacturers on a purchase order basis.

The facilities used by any contract manufacturer to manufacture OCA or any of our other product candidates are subject to inspection by the FDA and regulators in other jurisdictions. We are completely dependent on these third-party manufacturers for compliance with the requirements of U.S. and non-U.S. regulators for the manufacture of our finished products, including Ocaliva. If our manufacturers are unable to meet our requirements in accordance with our product specifications and applicable current Good Manufacturing Practices (“cGMP”) requirements, our products or product candidates will not be approved or, if already approved, may be subject to recall. In addition, if COVID-19 or related public health safety measures prevent the FDA, EMA or other relevant regulators from conducting manufacturing inspections or other regulatory activities with respect to manufacturing, it could significantly impact the ability of the FDA, EMA or such other regulators to timely review and process our regulatory submissions, which could have a material and adverse effect on our business and financial condition.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured our product candidates and products ourselves, including:

- the possibility that we are unable to enter into or renew our manufacturing agreements with third parties on acceptable terms, or at all;
- the possible termination, breach or non-performance by our third-party manufacturers of our manufacturing agreements based on factors beyond our control; and
- our inability to timely identify and qualify a replacement for any of our third-party manufacturers in the event any such third-party manufacturer fails to meet our product requirements or following the termination, expiration or nonrenewal of our agreements with such third-party manufacturer.

Any of these factors could disrupt the supply of our product candidates or approved products, cause us to incur higher costs, delay the approval of our product candidates or prevent or disrupt the commercialization of our approved products. Furthermore, if any of our product candidates, including OCA for liver fibrosis due to NASH, are approved and our contract manufacturers fail to deliver the required commercial quantities of API or finished product on a timely basis and at commercially reasonable prices and we are unable to find one or more replacement manufacturers capable of production at a substantially equivalent cost, in substantially equivalent volumes and quality and on a timely basis, we would likely be unable to meet demand for such product candidate following its approval and could lose potential revenue. It may take several years to establish an alternative long-term source of supply and to have any such new source approved by the regulatory authorities that regulate our products in the United States, Europe and our other target markets.

We depend on third-party vendors and CROs for certain of our clinical trial and product development activities. If any of these providers fail to comply with their contractual commitments or applicable regulatory obligations, including due to COVID-19 and related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions, business closures and disruptions and other public health safety measures, our business could be materially

and adversely affected. In addition, if we are unable to maintain our relationship with any one or more of these providers, we could experience a significant delay in both identifying another comparable provider and then contracting for its services, which could materially and adversely affect our clinical trial and product development efforts. We may be unable to retain an alternative provider on reasonable terms, or at all. Even if we locate an alternative provider, it is likely that such a provider will need additional time to respond to our needs and may not provide the same type or level of services as the original provider. Any third-party vendors and CROs that we retain are subject to the FDA's regulatory requirements and similar foreign standards and we do not have control over compliance with these regulations by these providers. The FDA, EMA and other relevant regulatory authorities enforce these regulations through periodic inspections of trial sponsors, principal investigators and trial sites. If these regulations are not adhered to by these providers, or if such providers fail to timely correct any non-compliance or if COVID-19 or related public health safety measures prevent the FDA, EMA or other relevant regulatory authorities from conducting inspections or other regulatory activities, the commercialization and development of our product candidates or approved products could be delayed, which could materially and adversely harm our business and financial condition.

***Even though we have received conditional approval of Ocaliva for PBC, we and our contract manufacturers are still subject to strict, ongoing regulatory requirements.***

Even though we have received conditional approval of Ocaliva for the treatment of PBC in combination with UDCA in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA, we and our contract manufacturers are subject to ongoing regulatory requirements relating to, among other things, Ocaliva's manufacturing, packaging, labeling and storage. In addition, we and our contract manufacturers and our contract manufacturers' facilities are required to comply with extensive FDA and EMA requirements and the requirements of other similar regulatory authorities, including requirements that quality control and manufacturing procedures conform to current cGMPs. As such, we and our contract manufacturers are subject to periodic cGMP inspections and other inspections and audits required by law or industry standard and must continue to expend time, money and effort to ensure compliance with applicable manufacturing, production and quality control requirements. We are also required to report certain adverse reactions and production problems, if any, to the FDA, EMA and other similar regulatory authorities and to comply with certain requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and generally must be consistent with the information in the product's approved label.

If a regulatory authority such as the FDA identifies previously unknown problems with one of our products, 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 one of our products, it may impose restrictions on that product or us, including requiring withdrawal of the product from the market. In addition, if we or our contract manufacturers, other third-party vendors or collaborators fail to comply with applicable regulatory requirements, a regulatory agency may, among other things:

- issue Form 483 notices or Warning Letters, in the case of the FDA, or similar notices, in the case of other regulatory agencies;
- mandate modifications to our promotional materials or require us to provide corrective information to healthcare practitioners;
- require us or our collaborators to enter into a consent decree or permanent injunction, which may include the imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- recall our products;
- suspend any of our ongoing clinical studies;
- impose administrative, civil or criminal penalties;

- withdraw regulatory approval or require changes to our product label, including the inclusion of additional warnings or changes to the approved indication;
- refuse to approve pending applications or supplements to approved applications filed by us or our collaborators;
- impose restrictions on our operations or those of our contract manufacturers, including costly new manufacturing requirements; or
- seize or detain products.

#### **Risks Related to the Commercialization of Our Products**

##### ***Sales of Ocaliva may be adversely affected by safety and labeling changes required by the FDA.***

In the course of our post-marketing pharmacovigilance activities, deaths have been reported in PBC patients with moderate or severe hepatic impairment. In an analysis performed by us and in consultation with the FDA, we concluded that certain of these patients were prescribed once daily doses of Ocaliva, which is seven times higher than the recommended weekly dose in such patients. As a result, in September 2017, we issued a DHCP letter and the FDA also subsequently issued its own drug safety communication to reinforce recommended label dosing. Both communications remind healthcare providers of the importance of the recommended reduced dosing of Ocaliva in PBC patients with moderate or severe hepatic impairment, while reiterating the importance of monitoring PBC patients for progression of their disease and the occurrence of liver-related adverse reactions. In February 2018, we announced that the Ocaliva label in the United States had been updated by the FDA to include a boxed warning and a dosing table that reinforced the then-existing dosing schedule for patients with Child-Pugh Class B or C or decompensated cirrhosis. In addition, the FDA issued an updated drug safety communication to accompany the revised label. We remain focused on the safety of all of the patients using Ocaliva within and outside of our ongoing clinical studies and have engaged with relevant regulatory authorities to ensure that the Ocaliva label sufficiently reinforces the importance of appropriate dosing in patients with advanced cirrhosis.

The FDA has notified us that, in the course of its routine safety surveillance, in May 2020 the FDA began to evaluate a newly identified safety signal, or NISS, regarding liver disorder for Ocaliva which the FDA classified as a potential risk. Pursuant to the FDA's guidance, this does not mean that the FDA has concluded that the drug has the listed risk or that the FDA has identified a causal relationship between Ocaliva and the potential risk. The FDA has informed us that its review of the NISS is focused on a subset of the cirrhotic, or more advanced, PBC patients who have taken Ocaliva. As part of our routine pharmacovigilance efforts, we have worked with the FDA to reconcile our internal safety database with the FDA Adverse Event Reporting System database and are now conducting a comprehensive assessment of all available data, including data from our completed clinical trials, blinded reviews of ongoing clinical trial data, unblinded reviews of ongoing clinical trial data by the data monitoring committee, post-marketing data and natural history data. Any safety concerns associated with Ocaliva, perceived or real, may adversely affect the successful development and commercialization of our product candidates and approved products, including Ocaliva, and materially and adversely affect our business.

***We are subject to uncertainty relating to pricing and reimbursement. Failure to obtain or maintain adequate coverage, pricing and reimbursement for Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, or our other future approved products, if any, could have a material adverse impact on our ability to commercialize such products.***

The availability and extent of coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products are key factors that will affect our future commercial prospects. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs they will cover and establish payment levels. Sales of our products depend and will depend substantially, both domestically and internationally, on the extent to which their cost will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations or reimbursed by government health administration authorities, private

health coverage insurers and other third-party payors. Accordingly, the coverage and reimbursement decisions of such governmental and private healthcare payors could reduce the demand for, or the price paid for, our products. If these payors do not consider our products to be cost-effective alone, or relative to other approved therapies, they may not cover our products or, if they do, they may apply utilization management restrictions, high patient cost-sharing obligations, or restrict the level of reimbursement.

Third-party payors are increasingly challenging the prices charged for pharmaceutical products, and many also limit reimbursement for newly-approved products and indications. Third-party payors often attempt to contain healthcare costs by demanding price discounts or rebates and limiting both the types and variety of drugs that they will cover and the amounts that they will pay for drugs. As a result, they may not provide adequate payment for our products. Similarly, the containment of healthcare costs has become a priority for federal and state governments and the pricing of pharmaceutical products has been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement, requirements for substitution of generic products and requirements to demonstrate a specific degree of improvement in terms of medical benefit compared to existing therapies. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could adversely affect our ability to successfully commercialize our products. In addition, we may be required to conduct post-marketing studies in order to demonstrate the cost-effectiveness of our products to payors' satisfaction. Such studies might require us to commit a significant amount of management's time and our financial and other resources and our products might not ultimately be considered cost-effective.

We do not know if Ocaliva for PBC will obtain and maintain broad acceptance from third-party payors in the jurisdictions in which it is, or may in the future be, approved. In addition, even if OCA for liver fibrosis due to NASH is approved, we do not know if it will obtain and maintain broad acceptance from third-party payors. The coverage determination process is a time-consuming and costly process that requires us to provide scientific and clinical support for the use of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH to each payor separately, with no assurance that coverage will be obtained or maintained. The market for a drug depends significantly on access to third-party payors' drug formularies, or lists of medications for which third-party payors provide coverage and reimbursement. Third-party payors may refuse to include a particular drug in their formularies or restrict patient access to a branded drug when a less costly generic equivalent or other alternative is available, even if not approved for the indication for which the branded drug is approved. Due to there being no uniform policy of coverage and reimbursement in the United States among commercial payors, coverage and reimbursement for pharmaceutical products may differ significantly from payor to payor. If we are unable to obtain and maintain adequate coverage from third-party payors, the adoption of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH by physicians and patients may be limited. This in turn could affect our ability to successfully commercialize Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH and have a material adverse impact our profitability, results of operations, financial condition and future success.

We cannot be certain that we will be able to obtain and maintain adequate coverage, pricing and reimbursement for our products, including Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, or our other future approved products, if any. If coverage or reimbursement is not available or is available on a limited basis, or if we are unable to obtain and maintain adequate pricing, we may not be able to successfully commercialize Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, or our other future approved products, if any.

***Legislative and regulatory healthcare reform may adversely affect our business.***

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (the "MMA") changed the way Medicare covers and pays for pharmaceutical products. The legislation established Medicare Part D, which expanded Medicare coverage for outpatient prescription drug purchases by the elderly but provided authority for limiting the number of drugs that will be covered in any therapeutic class. The MMA also introduced a new reimbursement methodology based on average sales prices for physician-administered drugs. Any negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act (collectively, the “ACA”), became law in the United States. Among other things, the purpose of the ACA was to reduce the cost of healthcare and substantially change the way healthcare is financed by both governmental and private insurers. The ACA requires discounts under the Medicare drug benefit program and increased the rebates paid by pharmaceutical companies on drugs covered by Medicaid. The ACA also imposes an annual fee, which increases each year, on sales by branded pharmaceutical manufacturers. Since its enactment, there have been a number of judicial, executive and legislative challenges to the ACA, including recent tax legislation that removed the financial penalties for people who do not carry health insurance and an Executive Order signed in October 2017 by President Trump directing federal agencies to modify how the ACA is implemented. There is still uncertainty over whether the ACA will undergo additional revisions, and we cannot predict the impact of any future modifications. Further, in December 2018, a federal district court in Texas ruled that the entire ACA was unconstitutional because it could not be considered an exercise of Congressional taxing authority following the repeal of the individual mandate penalties. In December 2019, a federal court of appeals upheld the district court's decision that the ACA individual mandate was unconstitutional absent financial penalties, but remanded the case back to the district court to determine whether the remaining provisions of the ACA were nonetheless valid. However, in March 2020, before the district court could rule on the ACA's remaining provisions, the U.S. Supreme Court agreed to review the case. Oral arguments likely will occur in late 2020, and a decision is expected sometime before June 2021. We cannot predict the outcome of this, or any other, litigation regarding the ACA or the impact it may have on our business.

Reimbursement in the European Union and many other territories must be negotiated on a country-by-country basis and in many countries a product cannot be commercially launched until reimbursement is approved. The timing to complete the negotiation process in each country is highly uncertain. Even after a price is negotiated, countries frequently request or require adjustments to the price and other concessions over time or require approvals regionally. Reimbursement agencies in Europe are often more conservative than those in the United States and the reimbursement process is often slower since reimbursement decisions are made on a country-by-country basis and may involve multiple government agencies in a given country. Prices for drugs in Europe are generally lower than in the United States and tend to decrease over time.

The United States and several other jurisdictions are considering, or have already enacted, a number of legislative and regulatory proposals to change their healthcare systems in ways that could affect our ability to sell our products profitably. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access to healthcare. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. We expect to experience pricing pressures in connection with the sale of Ocaliva and our other future approved products, if any, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative proposals. Pricing pressures recently experienced by the pharmaceutical industry may be further exacerbated by legislative and policy changes proposed or considered by the Trump administration and the United States Congress. For example, in June 2020, the Centers for Medicare & Medicaid Services released a proposed rule in a bid to update and expand key regulations governing the Medicaid Drug Rebate Program. If finalized as written, these changes will impact manufacturers' commercial strategies, government payor program liabilities, lifecycle management plans, patient assistance regimes, and other important considerations. Also, in September 2020, President Trump issued an Executive Order directing the Secretary of Health and Human Services to implement a rulemaking plan to test payment models for a most favored nation pricing system with respect to Medicare Part B and Part D prescription drugs. There have also been recent state legislative efforts to address drug costs, which have generally focused on increasing transparency around drug costs or limiting drug prices. In addition, it is possible that additional governmental action is taken to address pricing concerns arising in connection with COVID-19. We cannot predict the success or impact of any such current or future federal or state legislative efforts.

***Ocaliva and our other future approved products, if any, may not achieve broad market acceptance among physicians, patients and healthcare payors, and revenues generated from their sales may be limited as a result.***

The commercial success of Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, and our other future approved products, if any, will depend upon their acceptance among the medical community, including physicians,

healthcare payors and patients. In order for Ocaliva to be commercially successful for PBC, we need to demonstrate its utility as a cost-effective treatment for PBC patients who have an inadequate response to UDCA or who are unable to tolerate UDCA. Ocaliva also must be shown to be a safe and tolerable treatment in a commercial use setting as it is intended to be a lifetime therapy for patients eligible for treatment. We cannot be certain that Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, or our other future approved products, if any, will achieve an adequate level of acceptance among the medical community, including physicians, healthcare payors and patients.

In addition, we are closely evaluating the impact of COVID-19 on our ability to effectively market, sell and distribute Ocaliva for PBC. Our field-based employees are generally working remotely and are interacting with healthcare professionals via digital communication technologies such as, where appropriate, video conferences, emails and phone calls. Many of the healthcare professionals that we call on are working from home and facing additional demands on their time due to COVID-19. We are experiencing increased competition for virtual appointments with healthcare professionals, which may result in the cancellation of diagnostic, elective, specialty and other procedures and appointments to avoid non-essential patient exposure to medical environments and potential infection with COVID-19 and to focus limited resources and personnel capacity toward the treatment of COVID-19. Our increased utilization of virtual interactions and the reduced quantity of such interactions during the COVID-19 pandemic, may reduce the effectiveness of our sales personnel and negatively affect physician awareness and sales of Ocaliva. In the third quarter of 2020, we continued to see a lower level of prescriptions for new patients than we did prior to the outbreak of COVID-19. Although new patient starts currently account for only a small percentage of total Ocaliva prescriptions, our future sales of Ocaliva could be negatively impacted if prescriptions for new patients do not return to levels seen prior to the outbreak of COVID-19. It is also possible that COVID-19 may negatively affect our product sales in the future due to patient challenges in accessing healthcare providers, significant increases in unemployment and the resulting loss of individual health insurance coverage, and an inability to access government healthcare programs due to backlogs or other factors. As public health restrictions on travel and in-person interactions are modified or relaxed, we may continue to face challenges that limit our ability to fully resume in-person interactions, including the potential for additional outbreaks, limited access to personal protective equipment, the need to navigate varying restrictions for entering healthcare facilities and employee illness and childcare obligations during school closures. We also expect that the conversion of medical conferences to a virtual format may reduce our ability to appropriately disseminate scientific information and conduct disease state education. The long-term effects of COVID-19 are unknown and it is possible that following the pandemic, healthcare institutions could alter their policies with respect to in-person visits by pharmaceutical company representatives.

The degree of market acceptance of our approved products depends on a number of factors, including:

- limitations, warnings, precautions, boxed warnings, contraindications, restrictions or other statements contained in the product labels of our products, or any risk mitigation programs such as a REMS required for our products by the FDA, EMA or other relevant regulatory authorities;
- changes in the standard of care or availability of alternative therapies at similar or lower costs for the targeted indications for any of our products, such as UDCA for the treatment of PBC;
- limitations in the approved indications for our products;
- demonstrated and perceived clinical safety and efficacy compared to competitive products;
- a lack of adverse side effects, including deaths and other serious adverse events;
- sales, marketing and distribution support;
- the availability of reimbursement from managed care plans and other third-party payors;
- the timing of the market introduction of competitive products;
- the degree of cost-effectiveness;

- availability of alternative therapies at similar or lower cost, including generic and over-the-counter products;
- the extent to which our products are approved for inclusion on formularies of hospitals and managed care organizations;
- whether and to what extent our products are recommended under physician treatment guidelines for the treatment of the indications for which we have received regulatory approval;
- adverse publicity concerning our products or favorable publicity concerning competitive products;
- the convenience and ease of administration of our products;
- potential product liability claims; and
- the effects of COVID-19 and related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions, business closures and disruptions and other public health safety measures.

In addition, the potential market opportunity for Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, and our other future approved products, if any, is difficult to precisely estimate. For example, our estimates of the potential market opportunity for Ocaliva for PBC include a number of key assumptions related to prevalence rates, patients' access to healthcare, diagnosis rates and patients' response to or tolerance of Ocaliva, which are based on available literature and epidemiology research in PBC, our industry knowledge gained through market research and other methods, industry publications, third-party research reports and other surveys. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions. If any of these assumptions prove to be inaccurate, then the actual market for Ocaliva for PBC could be smaller than our estimates of our potential market opportunity. If the actual market opportunity for Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, or our other future approved products, if any, is smaller than we expect, our product revenue may be limited and our financial condition and results of operations may be materially and adversely affected.

If Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, or our other future approved products, if any, do not achieve an adequate level of acceptance among the medical community, including physicians, healthcare payors and patients, sufficient revenue may not be generated from these products and we may not become or remain profitable. In addition, our efforts to educate the medical community and third-party payors on the benefits of Ocaliva for PBC, OCA for liver fibrosis due to NASH, if approved, and our other future approved products, if any, may require significant resources and may never be successful.

***We have limited sales, marketing and distribution experience and we will need to continue to invest in significant additional resources to develop those capabilities or enter into acceptable third-party sales and marketing arrangements.***

We have limited sales, marketing and distribution experience as a commercial organization. Ocaliva is our first approved product and the commercial launch of Ocaliva for PBC was our first product launch. We are commercializing Ocaliva for PBC using a combination of our internal commercial organization and third-party distributors, depending on the jurisdiction. We are developing our commercialization strategy for OCA for liver fibrosis due to NASH, if approved, and have not yet decided on our commercialization strategy for OCA for other indications or for our other product candidates, in each case, if approved. To develop internal sales, distribution and marketing capabilities, we have invested, and expect to continue to invest, significant amounts of financial and management resources.

Recruiting and training a commercial organization is expensive, time-consuming and could delay any product launch. We can provide no assurance that we will correctly forecast the needs for growth of our commercial organization given our reliance on approvals from regulatory authorities for our product candidates. If the commercial launch of a product for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and

our investment could be lost if we cannot retain or reposition our sales and marketing personnel. For example, we expanded our commercial organization in anticipation of a potential U.S. commercial launch of OCA for liver fibrosis due to NASH. However, in June 2020, we received a CRL from the FDA with respect to our NDA for OCA for liver fibrosis due to NASH. Although we are in discussions with the FDA with respect to the potential resubmission of our NDA seeking accelerated approval of OCA for the treatment of liver fibrosis due to NASH, there is no assurance that we will be successful or that OCA will be approved for liver fibrosis due to NASH on an accelerated basis, or at all. Accordingly, our previously anticipated U.S. commercial launch of OCA for liver fibrosis due to NASH has been postponed, we do not expect to generate revenues for this indication until it has been approved, and we may incur significantly greater costs than previously anticipated in connection with the development of OCA for liver fibrosis due to NASH. On August 31, 2020, we adopted the 2020 Workforce Plan to reduce our workforce in light of the receipt of the CRL from the FDA. The 2020 Workforce Plan seeks to streamline our operations and reduce operating expenses, while maintaining the critical resources needed to continue to support the NASH and PBC clinical programs, pursue the approval of OCA for the treatment of liver fibrosis due to NASH and support our successful PBC business. The 2020 Workforce Plan will result in a workforce reduction of approximately 25%, or approximately 170 employees, and will largely affect the commercial infrastructure we developed in anticipation of a potential U.S. commercial launch of OCA for liver fibrosis due to NASH. The 2020 Workforce Plan was implemented during the third quarter of 2020, immediately after its announcement, and is expected to be substantially completed by the end of 2020.

For approved products where we decide to perform sales, marketing and distribution functions ourselves or through third parties, we could face a number of additional risks, including:

- we or our third-party sales collaborators may not be able to attract and build, or retain, an effective marketing or sales force;
- the cost of securing or establishing a marketing or sales force may exceed the revenues generated by our products; and
- our sales and marketing efforts may not be successful.

We may utilize the services of third-party collaborators in certain jurisdictions. We may have limited or no control over the sales, marketing and distribution activities of these third parties, and our future revenues may depend heavily on their success.

In addition, the effects of COVID-19 and related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions, business closures and disruptions and other public health safety measures may negatively impact our and our third-party collaborators' productivity, limit the conduct of business operations and impair our and our third-party collaborators' ability to execute our sales, marketing and distribution strategy.

***We could incur significant liability if it is determined that we have improperly promoted or are improperly promoting Ocaliva for PBC or any of our product candidates prior to their approval.***

Physicians are permitted to prescribe drug products for uses that are not described in the product's labeling and that differ from those approved by the FDA or other applicable regulatory agencies. Off-label uses are common across medical specialties. Although the FDA and other regulatory agencies do not regulate a physician's choice of treatments, the FDA and other regulatory agencies do restrict communications on the subject of off-label use. Companies are not permitted to promote drugs in a manner inconsistent with applicable regulatory guidance. The FDA, the U.S. Department of Justice ("DOJ") and other regulatory and enforcement authorities actively enforce laws and regulations prohibiting the improper promotion of approved products, as well as the promotion of products for which marketing approval has not been obtained. A company that is found to have improperly promoted off-label uses will be subject to significant liability, including civil and administrative remedies as well as criminal sanctions. A significant number of pharmaceutical companies have received inquiries or been the subject of investigations by various governmental authorities in the United States and abroad. Both federal and state governments have levied large civil and criminal fines against companies for alleged improper off-label promotion, as well as promotion that is determined to be false or misleading, even if related to approved indications.

While we have implemented a corporate compliance program based on what we believe are current best practices, we cannot provide any assurance that governmental authorities, including the DOJ, SEC or FDA, will find that our business practices comply with all current or future administrative or judicial interpretations of potentially applicable laws and regulations. In addition, government and regulatory agencies may hold us responsible for any actions by our sales representatives to the extent that they do not comply with applicable laws and regulations. If we fail to comply with any of these laws and regulations, we could be subject to a range of penalties, including the issuance of an untitled letter, a warning letter, injunction, seizure, criminal and significant civil penalties, fines, damages, disgorgement, curtailment or restructuring of our operations, exclusion, disqualification or debarment from participation in federally- or state-funded healthcare programs or other sanctions or litigation, any of which could have a material adverse impact on our business, financial condition and results of operations.

***If we market products in a manner that violates healthcare fraud and abuse laws, or if we violate government price reporting or physician payment disclosure laws, we may be subject to civil or criminal penalties.***

In addition to FDA restrictions on the marketing of pharmaceutical products, several other types of state and federal healthcare laws, commonly referred to as “fraud and abuse” laws, have been applied in recent years to restrict certain marketing practices in the pharmaceutical industry. Other jurisdictions including Europe have similar laws and are enacting more stringent regulations. These laws include false claims and anti-kickback statutes. If we market our products and our products are paid for by governmental programs, it is possible that some of our business activities could be subject to challenge under one or more of these laws.

Federal false claims laws generally prohibit anyone from knowingly and willingly presenting, or causing to be presented, any claims for the payment for goods (including drugs) or services to third-party payers (including Medicare and Medicaid) that are false or fraudulent. The federal civil monetary penalties statute, likewise, imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to generate business, including the purchase or prescription of a particular product covered by Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers or formulary managers on the other. Although there are several statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exemption or safe harbor. In addition, such exemptions and safe harbors are subject to change from time to time.

The Health Insurance Portability and Accountability Act of 1996 (as amended by the Health Information Technology for Economic and Clinical Health Act, “HIPAA”) created additional federal criminal statutes that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or obtain, by means of false or fraudulent pretenses, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. HIPAA also imposes significant requirements on the receipt and transfer of protected health information.

In addition, the federal transparency requirements under the Physician Payments Sunshine Act require certain manufacturers of drugs, including us, for which payment is available under certain federal healthcare programs annually to report information related to payments and other transfers of value to physicians and teaching hospitals, and physician ownership and investment interests. Beginning in 2021, the federal reporting requirements will also apply to payments and transfers of value to physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and anesthesiologist assistants, and certified nurse-midwives.

Finally, we must offer discounted pricing or rebates on Ocaliva and our future approved products, if any, under various federal and state healthcare programs, and report specific prices to government agencies under healthcare programs. The calculations necessary to determine the prices reported are complex and the failure to report prices accurately may expose us to significant penalties.

There are foreign and state law equivalents of these laws and regulations, such as anti-kickback, false claims, transparency and data privacy and security laws, to which we are currently and/or may in the future be subject. We may also be subject to foreign and state laws that require manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. Many of these laws differ from each other in significant ways, thus increasing the cost and complexity of our compliance efforts.

A number of pharmaceutical and other healthcare companies have been prosecuted under these laws for a variety of promotional and marketing activities, including providing free trips, free goods, sham consulting fees and grants and other monetary benefits to prescribers; reporting inflated average wholesale prices that were then used by federal programs to set reimbursement rates; engaging in improper promotional activities; and submitting inflated best price information to the Medicaid Drug Rebate Program to reduce liability for Medicaid rebates.

If we or our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including criminal and significant civil penalties, damages, fines, imprisonment, exclusion of products from reimbursement under United States federal or state healthcare programs, and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could materially and adversely affect our ability to operate our business and our financial results. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with these laws may prove costly.

***We may not be successful in establishing, implementing and maintaining development and commercialization collaborations, which could adversely affect our ability to develop certain of our product candidates and our financial condition and operating results. If any strategic collaborator fails to perform its obligations under, or terminates, its agreement with us, our business could be substantially harmed.***

Developing pharmaceutical products, conducting clinical trials, obtaining regulatory approval, expanding manufacturing capabilities and marketing approved products are expensive, complex and time-consuming undertakings. As a result, we have in the past entered into, and may in the future seek to enter into, collaborations with third parties upon whom we may rely for financial resources and for development, regulatory and commercialization expertise for selected products or product candidates and in selected jurisdictions. We may establish collaborations with respect to the development and commercialization of OCA in various jurisdictions and for our other product candidates. Additionally, we may enter into sales and marketing arrangements with third parties with respect to our approved products in all or certain jurisdictions.

Our collaborators may fail to develop our product candidates or effectively commercialize our products for a variety of reasons, including a lack of sufficient resources, a decision not to devote the necessary resources due to internal constraints, such as limited cash or human resources, a change in strategic focus, a failure to obtain the necessary regulatory approvals or business disruptions from COVID-19.

If we are unable to enter into new arrangements or maintain such arrangements on acceptable terms, or at all, we may be unable to effectively market and sell our products in certain of our target markets. We expect to face competition in seeking appropriate collaborators. Moreover, collaboration and similar arrangements are complex and time consuming to negotiate, document and implement and they may require substantial resources to maintain. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements for the development of our product candidates. When we collaborate with a third party for development and commercialization of a product candidate or approved product, we expect to relinquish some or all of the control over the future success of that product candidate or approved product to the third party. Our collaboration partner may not devote sufficient resources to development or

commercialization or may otherwise fail in their development or commercialization. The terms of any collaboration or other arrangement that we establish may not be favorable to us. In addition, any collaboration that we enter into may be unsuccessful. In some cases, we may be responsible for continuing preclinical and initial clinical development of a partnered product candidate or research program, and the payment we receive from our collaboration partner may be insufficient to cover the cost of this development. If we are unable to reach agreements with suitable collaborators, we may incur increased costs and we may be forced to limit the number of products or product candidates we can commercially develop or the territories in which we can commercialize them. If we fail to achieve successful collaborations, our operating results and financial condition could be materially and adversely affected.

***If we fail to develop OCA for additional indications such as NASH, our commercial opportunity will be limited.***

To date, we have focused the majority of our development efforts on the development of OCA. One of our strategies is to pursue clinical development of OCA for liver fibrosis due to NASH and other progressive non-viral liver diseases, to the extent that we have sufficient funding to do so.

PBC is an orphan disease and the potential market size for Ocaliva for PBC is relatively limited. Furthermore, because a significant proportion of PBC patients do not exhibit any symptoms at the time of diagnosis, PBC may be left undiagnosed for a significant period of time. Due to these factors, our ability to grow revenues will be dependent on our ability to increase market share and successfully develop and commercialize OCA for the treatment of additional indications. In particular, we believe that our future success will depend in large part on the results of our development of OCA for the treatment of NASH. Although NASH is believed to be one of the most prevalent chronic liver diseases worldwide, NASH may be left undiagnosed in patients for a long period of time and a definitive diagnosis of NASH is often based on a histological assessment of a liver biopsy, which impacts the ability to easily identify patients. Furthermore, even if we are successful in developing and obtaining marketing approval of OCA for the treatment of NASH, we may not be commercially successful.

The completion of development, securing of approval and commercialization of OCA for additional indications such as liver fibrosis due to NASH will require substantial additional funding, is subject to numerous risks and we may not be successful. We cannot provide you any assurance that we will be able to successfully advance any of these indications through the development process. Even if we receive regulatory approval to market OCA for the treatment of liver fibrosis due to NASH or any other additional indications, we cannot assure you that any such additional indications will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. If we are unable to successfully develop and commercialize OCA for liver fibrosis due to NASH or other additional indications, our commercial opportunity will be limited and our business prospects will suffer.

#### **Risks Related to Our Business and Strategy**

***We depend on third-party contractors for a substantial portion of our operations and may not be able to control their work as effectively as if we performed these functions ourselves.***

We outsource and plan to continue to outsource substantial portions of our operations to third-party service providers, including CROs for certain of our clinical trial and product development activities, contract manufacturers for the production of API and finished drug product for our commercial sales and for our clinical trials and preclinical studies. We will likely also use the services of third-party vendors in connection with our future commercialization activities, including product sales, marketing and distribution. Our agreements with third-party service providers are typically on a study-by-study and/or project-by-project basis. Typically, we may terminate these agreements with notice and are responsible for the supplier's previously incurred costs. In addition, a number of third-party service providers that we retain will be subject to the FDA's and EMA's regulatory requirements and similar standards outside of the United States and Europe and we do not have control over compliance with these regulations by these providers. If these providers do not adhere to applicable governing practices and standards, the commercialization of Ocaliva and our other approved products, if any, and the development of OCA and our other product candidates could be delayed or stopped, which could severely harm our business and financial condition.

Because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves the risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. There are a limited number of third-party service providers that have the specialized expertise required to achieve our business objectives. Identifying, qualifying and managing the performance of third-party service providers can be difficult, time-consuming and cause delays in our development programs. Despite our growth, we have limited internal resources available to identify and monitor third-party service providers. To the extent we are unable to identify, retain and successfully manage the performance of third-party service providers, our business may be materially and adversely affected. We may further be subject to the imposition of civil or criminal penalties if their conduct violates applicable law.

Our third-party service providers generally are not prohibited from providing their services to other biopharmaceutical companies, including companies that currently or may in the future compete with us. For example, certain of our third-party service providers and consultants may be able to develop intellectual property to which we do not have rights under our agreements and that may eventually be used to develop products that compete with our products. Although we generally have confidentiality and non-disclosure agreements in place with our third-party service providers and consultants, such third parties may be able to provide services to other companies without violating the terms of our agreements. In addition, although we may seek to enter into non-compete arrangements with our key third-party service providers, such arrangements are difficult to negotiate and we may be unable to successfully enter into or enforce such arrangements.

The effects of COVID-19 and related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions, business closures and disruptions and other public health safety measures may negatively impact our and our third-party service providers' productivity, limit the conduct of business operations and impair our and our third-party service providers' ability to conduct operations.

***We face rapid technological change and competition from other biotechnology and pharmaceutical companies. Our operating results will suffer if we fail to compete effectively.***

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. We have competitors in the United States, Europe and other jurisdictions, including major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical and generic drug companies and universities and other research institutions. Many of our competitors have financial, sales and marketing, manufacturing and distribution, legal, regulatory and product development resources substantially greater than ours. Large pharmaceutical companies, in particular, have extensive experience in research, clinical testing, obtaining regulatory approvals, recruiting patients and manufacturing pharmaceutical products. These companies also have significantly greater sales and marketing capabilities and often have collaborative arrangements in our target markets. Established pharmaceutical companies may also invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our products or product candidates obsolete. As a result of all of these factors, our competitors may succeed in obtaining patent protection and/or FDA, EMA or other regulatory approval or discovering, developing and commercializing drugs for the diseases that we are targeting before we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies.

Some of the pharmaceutical and biotechnology companies that we may compete with include 89bio, Inc., AbbVie Inc., Acorda Therapeutics, Inc., Affimune Limited, Akcea Therapeutics, Inc., Akerio Therapeutics, Inc., Albireo Pharma, Inc., Altimune, Inc., Arrowhead Pharmaceuticals, Inc., AstraZeneca plc, Blade Therapeutics, Inc., Boehringer Ingelheim GmbH, Bristol-Myers Squibb Company, Can-Fite BioPharma Ltd., Cirus Therapeutics, Inc., Corcept Therapeutics Incorporated, CymaBay Therapeutics, Inc., Dr. Falk Pharma GmbH, Durect Corporation, Eli Lilly and Company, Enanta Pharmaceuticals, Inc., Forma Therapeutics, Inc. Galectin Therapeutics Inc., Galecto Biotech AB, Galmed Pharmaceuticals Ltd., Genfit SA, Genkyotex, Gilead Sciences, Inc., GlaxoSmithKline plc, GRI Bio, Inc., Hanmi Pharmaceutical Co., Ltd., Hepion Pharmaceuticals, Inc., HighTide Therapeutics Inc., Immuron Limited, Inventiva, Ionis Pharmaceuticals, Inc., Kowa Company, Ltd., Lipocine Inc., Madrigal Pharmaceuticals, Inc., MediciNova, Inc., Metacrine, Inc., Mitsubishi Tanabe Pharma Corporation, Nash Pharmaceuticals Inc., NGM Biopharmaceuticals, Inc., NorthSea Therapeutics B.V.,

Novartis AG, Novo Nordisk A/S, NuSirt Biopharma, Inc., Oramed Pharmaceuticals Inc., Pfizer Inc., Poxel SA, Sagimet Biosciences Inc., Second Genome, Inc., Sinew Pharma Inc., Terns Pharmaceuticals, Inc., Theratechnologies, Inc., Viking Therapeutics, Inc., Yagrit International Ltd and Zydus Pharmaceuticals (USA) Inc. Ocaliva competes with UDCA (or ursodiol), a first-line therapy approved for the treatment of PBC that is available generically at a significantly lower cost than Ocaliva. Although we have a license to develop and commercialize bezafibrate in the United States, bezafibrate has been studied in multiple clinical trials for the treatment of liver diseases including PBC and NASH outside of the United States. Genfit is studying elafibranor for the treatment of PBC. Gilead Sciences, Inc. is studying firsocostat, a small molecule allosteric inhibitor that acts at the protein-protein homodimer interface of acetyl-CoA carboxylases and cilofexor, an FXR agonist, in NASH patients. Gilead Sciences, Inc. is also studying a number of compounds in other liver diseases including PBC.

In addition, many universities and private and public research institutions may become active in our target disease areas. The results from our clinical trials and the approval of Ocaliva for PBC have brought more attention to our targeted indications and bile acid chemistry. As a result, we believe that additional companies and organizations may seek to compete with us in the future. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis, technologies and drug products that are more effective or less costly than OCA or any other product candidates that we are currently developing or that we may develop, which could render our products or product candidates obsolete and noncompetitive. Our ability to compete may also be affected because, in many cases, insurers or other third-party payors seek to encourage the use of generic products.

Off-label uses of other potential treatments may limit the commercial potential of our products and product candidates, especially given the pricing of Ocaliva and the anticipated pricing for our product candidates. For example, while fibrates are not approved for use in PBC, off-label use of fibrate drugs has been reported. In NASH, a number of treatments, including vitamin E (an antioxidant), insulin sensitizers (e.g., metformin, pioglitazone), antihyperlipidemic agents (e.g., gemfibrozil), pentoxifylline and UDCA, are used off-label. Although none of these treatments have been clearly shown in clinical trials to alter the course of the disease, in a previous study conducted by the NASH Clinical Research Network, improvements in certain histological measures of NASH were reported with vitamin E and pioglitazone.

We believe that our ability to successfully compete will depend on, among other things:

- the results of our and our strategic collaborators' clinical trials and preclinical studies;
- our ability to recruit, enroll and retain patients for our clinical trials;
- the efficacy, safety and tolerability of Ocaliva, OCA for liver fibrosis due to NASH, if approved, and our other future approved products, if any;
- the speed at which we develop our product candidates;
- our ability to design and successfully execute appropriate clinical trials;
- our ability to maintain productive relationships with regulatory authorities;
- the timing and scope of regulatory approvals, if any;
- our ability to commercialize and market Ocaliva, OCA for liver fibrosis due to NASH, if approved, and our other future approved products, if any;
- the price of our products;
- our ability to obtain adequate levels of reimbursement under private and governmental health insurance plans, including Medicare;

- our ability to protect our intellectual property rights related to our products;
- our ability to manufacture and sell commercial quantities of Ocaliva, OCA for liver fibrosis due to NASH, if approved, and our other future approved products, if any, to the market; and
- the acceptance of our products by physicians and other healthcare providers.

If our competitors market products that are more effective or safe or less expensive than our products or that reach the market sooner than our products, we may not achieve commercial success. In addition, the biopharmaceutical industry is characterized by rapid technological change. Because our research approach integrates many technologies, it may be difficult for us to stay abreast of the rapid changes in other technologies. If we fail to stay at the forefront of technological change, we may be unable to compete effectively. Technological advances or products developed by our competitors may render our technologies, products or product candidates obsolete, less competitive or not economical.

***A variety of risks associated with our international business operations and our planned international business relationships could materially and adversely affect our business.***

We have formed a number of subsidiaries in jurisdictions outside of the United States in connection with or in anticipation of our commercial or other business activities in those jurisdictions. We are commercializing Ocaliva for PBC using a combination of our internal commercial organization and third-party distributors, depending on the jurisdiction. Our variety of international operations and business relationships subject us to additional risks that may materially and adversely affect our business and ability to attain or sustain profitability, including:

- the enhanced anti-bribery and anti-corruption regimes now implemented in most European Union member states and elsewhere, including the UK Bribery Act (thought to be the strictest anti-bribery legislation in the world) and the escalation of investigations and prosecutions pursuant to such laws;
- compliance with complex import and export control laws;
- restrictions on direct investments by foreign entities and country-specific trade restrictions;
- differing regulatory requirements for drug approvals across our various international markets and as a result the potential inability to obtain necessary foreign regulatory, pricing or reimbursement approvals for our products in a timely manner, or at all;
- variances in payment terms and uncertainty regarding the collectability of accounts receivable from our counterparties in our international business;
- difficulties in staffing and managing international operations;
- the potential for reduced protection for our intellectual property rights;
- the potential for third-party patent rights in countries outside of the United States;
- the impact of parallel trade within the European Union which occurs when a parallel trader purchases goods in one country where the price is lower and sells it into another country where the price is higher;
- unexpected changes in tariffs, trade barriers and regulatory requirements and the imposition of governmental controls;
- economic weakness, including inflation, or political instability, particularly in non-U.S. economies and markets, including countries in Europe;

## [Table of Contents](#)

- compliance with tax, employment, immigration and labor laws applicable to our employees working or traveling abroad;
- compliance with data protection laws, including regimes relating to cross-border transfer mechanisms;
- taxes in other countries;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other transactional risks incident to doing business in foreign countries;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geo-political actions, including war and terrorism, global health emergencies, such as COVID-19, natural disasters, including earthquakes, volcanoes, typhoons, floods, hurricanes and fires, or social unrest; and
- increasingly complex standards for complying with foreign laws and regulations that may differ substantially from country to country and may conflict with corresponding U.S. laws and regulations.

On January 31, 2020, the United Kingdom left the European Union, in what is often referred to as Brexit. Since the initial European Union referendum in June 2016, negotiations for Brexit have caused political and economic uncertainty, including in the regulatory framework applicable to the operations of biotechnology and pharmaceutical companies, and this uncertainty may persist well beyond the end of the Brexit transition period on December 31, 2020. Brexit could, among other outcomes, disrupt the free movement of goods, services and people between the United Kingdom and the European Union, result in disruption to, and uncertainty regarding the application and interpretation of, national and international laws and regulations and introduce other legal and regulatory complexities. For example, because a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, Brexit could materially change the regulatory regime applicable to our operations, including with respect to Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH and our other product candidates. Such outcomes could make it more difficult and expensive for us to do business in Europe, complicate our clinical, manufacturing and regulatory strategies and impair our ability to obtain and maintain regulatory approval for, and, if approved, commercialize, our products and product candidates in Europe. In addition, our ability to continue to conduct our international operations out of the United Kingdom, where the headquarters for our international operations is located, may be materially and adversely affected. While we have undertaken a number of Brexit-related contingency planning initiatives, the full potential financial, legal, regulatory and other implications of Brexit are uncertain and we cannot make any assurances regarding the extent to which our business may be adversely affected thereby.

In addition, we are subject to the anti-bribery and anticorruption laws of the United States, as well as of foreign jurisdictions where we operate, including the U.S. Foreign Corrupt Practices Act and the UK Bribery Act. Generally, these laws prohibit paying or offering anything of value to a foreign government official for the purpose of obtaining or retaining business but they can also have a much wider, extraterritorial scope, as is the case with the UK Bribery Act (thought to be the strictest anti-bribery legislation in the world). U.S. and foreign regulators have increased their enforcement of anti-bribery and anticorruption laws in recent years, and failure to comply with these laws could result in various adverse consequences, including:

- the possible delay in approval or refusal to approve our product candidates;
- recalls, seizures or withdrawal from the market of an approved product;
- disruption in the supply or availability of our products or suspension of export or import privileges;

- the imposition of civil or criminal sanctions;
- the prosecution of executives overseeing our international operations; and
- damage to our reputation.

Any significant impairment of our ability to develop our product candidates or sell our approved products outside of the United States could adversely impact our business and financial results.

***Our business and operations would suffer in the event of system failures, data breaches or violations of data protection laws.***

We are increasingly dependent on information technology systems and infrastructure, including mobile technologies, to operate our business. In the ordinary course of our business, we collect, process, store and transmit large amounts of confidential information, including intellectual property, proprietary business information and personal information. It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such information. The size and complexity of our information technology systems, and those of third-party vendors with whom we contract, and the volume of data we retain, make such systems potentially vulnerable to breakdown, malicious intrusion, security breaches and other cyber-attacks. Information security risks have significantly increased in recent years in part due to the proliferation of new technologies and the increased sophistication and activities of organized crime, hackers, terrorists and other external parties, including foreign state actors. Our information security systems and those of our third party vendors are subject to laws and regulations, or may become subject to new laws and regulations, requiring that we enact certain measures to protect the privacy and security of certain information we collect or use in our business. A security breach or privacy violation that leads to disclosure or modification of, or prevents access to, personal information or other protected information, whether caused by internal or external parties, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to notification requirements under certain agreements with third parties, 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 information, resulting in increased costs or loss of revenue. Similarly, the loss of clinical trial data from completed or ongoing or planned clinical trials could prevent us from obtaining regulatory approval or delay our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we are unable to prevent such security breaches or privacy violations or implement satisfactory remedial measures, our operations could be disrupted, and we may suffer loss of reputation, financial loss and be subject to regulatory fines and penalties. In addition, these breaches and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. Moreover, the reliance on remote working technologies by our employees and third party partners due to COVID-19 and related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions and other public health safety measures and the prevalent use of mobile devices that access confidential and personal information increases the risk of data security breaches, which could lead to the loss of confidential information, personal information, trade secrets or other intellectual property. As cyber threats continue to evolve, we may be required to expend significant additional resources to continue to modify or enhance our protective measures or to investigate and remediate any information security vulnerabilities. While we have implemented security measures to protect our data security and information technology systems, such measures may not prevent such events. Significant disruptions of our information technology systems or breaches of data security could have a material adverse effect on our business, financial condition and results of operations.

In the United States, numerous federal and state laws, including, without limitation, HIPAA state security breach notification laws, state health information privacy laws and federal and state consumer protection laws, govern the collection, use, disclosure and storage of personal information as well as consumer rights with regard to such information. For example, California recently passed the California Consumer Privacy Act of 2018, which became effective on January 1, 2020. Various foreign countries where we may process personal information also have, or are developing, privacy and data protection laws governing the collection, use, disclosure and storage of personal information.

In July 2016, U.S. and European Commission officials adopted a framework called the European Union-U.S. Privacy Shield (the “EU Privacy Shield”) to govern transfers of personal data from the European Economic Area (“EEA”) to the

U.S. We adopted the European Union-U.S. Privacy Shield and have certified to its requirements since October 2016. We also adopted the Swiss-U.S. Privacy Shield (the “Swiss Privacy Shield”) in order to legitimize the transfer of personal data from Switzerland to the U.S. In May 2018, the General Data Protection Regulation (the “GDPR”) took effect in the EEA. The GDPR imposes more stringent data protection requirements, and provides for greater penalties for noncompliance, than previous EEA data protection legislation. In addition, although we have implemented certain measures as a result of Brexit to allow for the transfer of personal data between EEA member states and the United Kingdom, we may need to develop additional mechanisms to permit for the transfer of this data. Implementation of the GDPR and other changes in privacy and data protection laws or regulations could require changes to certain of our business practices, thereby increasing our costs. While we are engaging in activities to comply with the GDPR requirements and other data protection laws, we may be unsuccessful in these efforts.

On July 16, 2020, the Court of Justice of the European Union (“CJEU”) invalidated the EU Privacy Shield as a data transfer mechanism for transferring personal data from the EEA to the U.S., effective immediately. On September 20, 2020, the Swiss Federal Data Protection and Information Commissioner invalidated the Swiss Privacy Shield. Therefore, the EU Privacy Shield and the Swiss Privacy Shield no longer qualify as appropriate safeguards for the transfer of personal data from the EEA or Switzerland to the U.S. and transfers made under those frameworks could attract regulatory scrutiny and penalties for non-compliance. There is no “grace period” to allow organizations to implement an alternative data transfer mechanism to the EU Privacy Shield and the Swiss Privacy Shield. While the European Commission approved Standard Contractual Clauses (“SCCs”) and Binding Corporate Rules remain a valid mechanism to transfer personal data to third countries outside the EEA and Switzerland, the CJEU’s ruling has also imposed enhanced due diligence obligations on organizations acting as data exporters and relying on SCCs to ensure that the laws of the country to which personal data is transferred offers a level of data protection that is essentially equivalent to the EEA. In addition, the European Commission is in the process of updating the SCCs to align them with the GDPR. As a result of the CJEU’s ruling, the status of the transfers of personal data from the EEA or Switzerland to the U.S. is currently subject to significant regulatory uncertainty and we are actively monitoring developments in this area. To the extent we are not able to employ suitable data transfer mechanisms to facilitate international transfers of data, our ability to conduct our business may be materially adversely impacted.

The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing amount of focus on privacy and data protection issues that may affect our business. There is a degree of uncertainty associated with the legal and regulatory environment around privacy and data protection laws, which continue to develop in ways we cannot predict, including with respect to evolving technologies, such as cloud computing. Privacy and data protection laws may be interpreted and applied inconsistently from country to country and impose inconsistent or conflicting requirements. As a result, our practices may not comply in the future with all such privacy and data protection laws. Varying jurisdictional requirements could increase the costs and complexity of compliance or require us to change our business practices in a manner adverse to our business. A determination that we have violated any privacy or data protection laws could result in significant damage awards, fines and other penalties that could, individually or in the aggregate, materially harm our business and reputation. For example, administrative fines of up to the greater of €20 million or 4% of our global turnover may be imposed for breaches of the GDPR; we may also be liable should any individual who has suffered financial or non-financial damage arising from our infringement of the GDPR exercise his or her right to receive compensation against us.

In addition, our marketing activities and the marketing activities of any third parties on which we rely are subject to various regulations, including privacy and data protection laws, consumer protection laws and competition laws. Such laws may impair our ability, or the ability of third parties on which we rely, to collect information. Such regulations may have a negative effect on businesses and may increase the potential civil liability and cost of operating our business.

***We have significantly expanded our operations and plan to continue our expansion to support our future development strategy for OCA for indications other than PBC, including liver fibrosis due to NASH. We may experience difficulties in managing our significant growth.***

We have significantly expanded our operations, including the size of our employee base, as we pursue our future development and commercialization strategy. As we advance our preclinical and clinical development programs for OCA and our other product candidates, seek regulatory approval in the United States and elsewhere and pursue our

commercialization strategy, we may need to increase our product development, scientific, commercial and administrative headcount. Such an evolution may impact our strategic focus and our deployment and allocation of resources. Our management, personnel and systems may experience difficulty in adjusting to our growth and strategic focus.

We may also anticipate needs for growth that do not materialize. For example, we expanded our commercial organization in anticipation of a potential U.S. commercial launch of OCA for liver fibrosis due to NASH. However, in June 2020, we received a CRL from the FDA with respect to our NDA for OCA for liver fibrosis due to NASH. Although we are in discussions with the FDA with respect to the potential resubmission of our NDA seeking accelerated approval of OCA for the treatment of liver fibrosis due to NASH, there is no assurance that we will be successful or that OCA will be approved for liver fibrosis due to NASH on an accelerated basis, or at all. On August 31, 2020, we adopted the 2020 Workforce Plan to reduce our workforce in light of the receipt of the CRL from the FDA. The 2020 Workforce Plan seeks to streamline our operations and reduce operating expenses, while maintaining the critical resources needed to continue to support the NASH and PBC clinical programs, pursue the approval of OCA for the treatment of liver fibrosis due to NASH and support our successful PBC business. The 2020 Workforce Plan will result in a workforce reduction of approximately 25%, or approximately 170 employees. The 2020 Workforce Plan was implemented during the third quarter of 2020, immediately after its announcement, and is expected to be substantially completed by the end of 2020. We can provide no assurance that we will correctly forecast the needs for growth given our reliance on approvals from regulatory authorities for our product candidates.

In addition, in order to continue to meet our obligations as a public company and to support our anticipated longer-term growth, we may need to increase our general and administrative capabilities. We have also expanded our operations geographically and formed a number of subsidiaries outside of the United States. In addition to our U.S. offices, we have an office in London, which serves as the headquarters for our international operations, and regional offices in a number of other countries, and we may further expand our geographical footprint. Our management, personnel and systems may not be adequate to support this future growth. Furthermore, we may face a number of complexities, such as being subject to national collective bargaining agreements for employees, in some of the countries in which we operate.

Our need to effectively manage our operations, growth and various projects requires that we:

- successfully attract and recruit new employees or consultants with the expertise and experience we require in the United States, Europe and other jurisdictions;
- develop, expand or adjust our commercial infrastructure;
- manage our clinical programs effectively, which are often conducted at numerous domestic and international clinical sites, and advance our other development efforts; and
- continue to improve our operational, financial and management controls, reporting systems and procedures.

If we are unable to successfully manage our growth and the increased complexity of our operations, our business may be materially and adversely affected.

***We may not be able to manage our business effectively if we are unable to attract and retain key personnel and consultants.***

We may not be able to attract or retain qualified personnel and consultants due to the intense competition for such individuals among biotechnology, pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel and consultants to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development and commercial objectives, our ability to raise additional capital and our ability to implement our business strategy.

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the development, regulatory, commercialization and business development expertise of Dr. Mark Pruzanski, our co-founder, president and chief executive officer, and the other members of our executive team, as well as other key

employees and consultants. If we lose one or more of our executive officers or other key employees or consultants, our ability to implement our business strategy successfully could be seriously harmed. Any of our executive officers or other key employees or consultants may terminate their employment at any time and replacing such individuals may be difficult and time-consuming because of the limited number of individuals in our industry with the necessary breadth of skills and experience. Competition to hire and retain employees and consultants from this limited pool is intense, and we may be unable to hire, train, retain or motivate such individuals.

We also have key advisors and consultants who assist us in operating our business. These advisors are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us and such individuals typically will not enter into non-compete agreements with us. If a conflict of interest arises between their work for us and their work for another entity, we may lose their services. In addition, our advisors may assist other companies that compete with us.

***Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading, which could significantly harm our business.***

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with the regulations of the FDA, the SEC or other domestic or foreign regulators, provide accurate information to the FDA, the SEC or other domestic or foreign regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive regulation in the United States and abroad intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. Such laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. Misconduct and misappropriation of confidential information by our employees or third parties may also include improper trading in our securities, which may harm our reputation and result in enforcement actions against us. We have adopted a global code of business conduct and implemented a corporate compliance program, 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 inquiries, investigations or other actions or lawsuits stemming from a failure to comply with applicable laws or regulations. The outcome of any such inquiry, investigation, action or lawsuit could have a significant negative impact on our business, including as a result of the imposition of significant fines or other sanctions. In addition, the institution of any such inquiry, investigation, action or lawsuit could negatively impact the market price of our securities.

***We face potential product liability exposure, and if successful claims are brought against us, we may incur substantial liability for our products or product candidates and may have to limit or suspend their use.***

The use of our product candidates in clinical trials and the sale of any products for which we have obtained or may obtain marketing approval, such as Ocaliva for PBC, expose us to the risk of product liability claims. Product liability claims may be brought against us or our collaborators by participants enrolled in our clinical trials, patients, healthcare providers or others. If we cannot successfully defend ourselves against any such claims, we may incur substantial liabilities. Regardless of their merit or eventual outcome, product liability claims may result in:

- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire clinical trial programs;
- costs of related litigation;
- substantial monetary awards to patients or other claimants;
- decreased demand for our products and loss of revenues;

- impairment of our business reputation;
- diversion of management and scientific resources from our business operations; and
- the inability to develop and commercialize our products and product candidates or the withdrawal of our products from the market.

We have obtained limited product liability insurance coverage. Our insurance coverage may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to product liability. Large judgments have been awarded in class action lawsuits based on the unanticipated side effects of drug products. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash resources and adversely affect our business.

### **Risks Related to Our Intellectual Property**

***It is difficult and costly to protect our proprietary rights, and we may not be able to ensure their protection. If our patent position does not adequately protect our products such as Ocaliva and product candidates such as OCA for liver fibrosis due to NASH, others may compete against us more directly, which could harm our business, possibly materially.***

Our commercial success will depend in part on our ability to obtain and maintain patent, trademark and trade secret protection covering Ocaliva, OCA for liver fibrosis due to NASH, if approved, and our other product candidates, as well as our ability to successfully defend our intellectual property against third-party challenges. Our ability to stop third parties from making, using, selling, offering to sell or importing our products is dependent upon the extent to which we have regulatory exclusivity or intellectual property-based exclusivity rights under valid and enforceable patents or other intellectual property that cover our products. If we fail to obtain and maintain adequate intellectual property protection, we may not be able to prevent third parties from launching generic versions of our products, from using our proprietary technologies or from marketing products that are very similar or identical to ours. For example, we have received paragraph IV certification notice letters from several generic drug manufacturers indicating that each such company has submitted to the FDA an ANDA seeking approval to manufacture and sell a generic version of our 5 mg and 10 mg dosage strengths of Ocaliva (obeticholic acid) for PBC prior to the expiration of certain patents protecting Ocaliva. We have initiated patent infringement suits against each of these generic drug manufacturers in the United States District Court for the District of Delaware. While we intend to vigorously defend and enforce our intellectual property rights protecting Ocaliva, we can offer no assurance as to when the lawsuits will be decided, or whether the lawsuits will be successful. If a generic equivalent of Ocaliva is approved and enters the market before the expiration of our patents protecting Ocaliva, our business may be materially and adversely affected. See Note 18 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q for more information.

The patent positions of pharmaceutical companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the United States or in foreign jurisdictions, and the legal standards relating to the patentability, validity and enforceability of pharmaceutical patents are evolving. Changes in either the patent laws or in interpretations of patent laws in U.S. and foreign jurisdictions may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the patents that we currently own or that may issue from the applications we have filed or may file in the future or those that we may license from third parties. Additionally, our currently pending or future patent applications may not result in issued patents, and any term extensions or reissues that we seek may not be granted. Further, if any patents we obtain or license are deemed invalid or unenforceable, it could impact our ability to commercialize or license our technology or we may not be able to prevent third parties from launching generic versions of our products, or from developing or marketing products that are similar or identical to ours.

There have been numerous changes to the patent laws that may have a significant impact on our ability to protect our technology and enforce our intellectual property rights. In September 2011, the America Invents Act was signed into law.

The final substantive provisions of the America Invents Act became effective in March 2013. The America Invents Act included a number of significant changes to U.S. patent law that affect the way patent applications are filed, prosecuted and litigated, including, among other things, changing from a “first to invent” to a “first inventor to file” system and creating processes, such as Inter Partes Review (“IPR”) and other post-grant review processes, that permit third parties to challenge the validity of granted patents before the Patent Trial and Appeal Board of the U.S. Patent and Trademark Office (the “USPTO”). The IPR process, for example, permits any person to challenge the validity of a patent on the grounds that it was anticipated or made obvious by prior art. The America Invents 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, financial condition, results of operations and growth prospects.

Others have filed, and in the future, are likely to file, patent applications covering products and technologies that are similar or competitive to ours, or may be important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in infringement, interference, derivation, opposition, nullity, invalidity or other similar proceedings before U.S. or non-U.S. patent offices or courts.

The degree to which our patents protect our products may be limited due to a number of factors. For example:

- others may be able to develop and market products that are similar to our products or product candidates but not covered by the claims of our patents;
- we might not have been the first to conceive of the inventions covered by our patents or pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- patents that we obtain may not provide us with competitive advantages or exclusivity in a particular product area or indication or for the length of time we have anticipated; or
- the patents of others may have an adverse effect on our business.

We are the owner of record of numerous issued U.S. and non-U.S. patents and patent applications with claims directed to pharmaceutical compounds, pharmaceutical compositions, formulations, methods of making these compounds and methods of using these compounds in various indications.

Our issued patents for OCA are expected to expire between 2022 and 2036 if the appropriate maintenance, renewal, annuity, or other government fees are paid. Without patent protection, including patent protection covering the composition of matter, methods of using and formulations of our products and product candidates, our ability to stop others from making, using, selling, offering to sell or importing our products and product candidates may be limited.

Due to the patent laws of a specific country in which we are seeking patent protection, the decisions of a patent examiner in a specific country in which we are seeking patent protection or our own filing strategies, we ultimately may not obtain patent coverage for all of our products and product candidates for which we have filed a patent application. While we regularly pursue patent protection in the United States and other countries to obtain claim coverage for our inventions, we cannot be certain that such patent rights will be granted or that the scope of any patent granted will prevent third parties from making, using, selling, offering for sale or importing the same or similar products.

***If we do not obtain protection under the Hatch-Waxman Act in the United States (and similar legislation outside of the United States) extending the terms of our patents and/or providing data or other exclusivity for our products and product candidates, our business may be materially harmed.***

Depending upon the timing, duration and specifics of FDA marketing approval of our products, U.S. patents may be eligible for a limited extension of patent term under the Drug Price Competition and Patent Term Restoration Act of 1984 (the “Hatch-Waxman Act”). The Hatch-Waxman Act permits an extension of patent term for one patent of up to five years as compensation for patent term lost during product development and the FDA regulatory review process, so long as the total period of patent term extension does not exceed 14 years from the date of approval. However, an extension may not be granted because of, for example, failure to apply within applicable deadlines, failure to apply prior to expiration of relevant patents or failure to satisfy applicable requirements. Moreover, the applicable time period or scope of patent protection afforded could be less than what is requested. If we are unable to obtain patent term extension or the term of any such extension is less than what we request, the period during which we will have the right to exclusively market our products may be shorter than anticipated, our competitors may obtain approval of competing products following our patent expiration and our revenue could be reduced, possibly materially.

In October 2020, the USPTO granted to the Company a reissue patent, U.S. Patent No. RE 48,286 (the “‘286 Patent”). By operation of law, our primary composition of matter patent protecting Ocaliva, U.S. Patent No. 7,138,390 (the “‘390 Patent”), was withdrawn and replaced by the ‘286 Patent, which contains composition of matter claims to OCA. The ‘286 Patent will be substituted in any litigation where the ‘390 Patent was asserted. The ‘286 Patent, like its predecessor the ‘390 Patent, was scheduled to expire in 2022. In light of the U.S. marketing approval of Ocaliva for PBC in May 2016, and pursuant to the Hatch-Waxman Act, we previously applied for an extension of the patent term for the ‘390 Patent in the U.S. seeking to extend the term of the ‘390 Patent into 2027. In November 2020, the Company was informed by the USPTO that its petition for a five-year patent term extension had been granted. Accordingly, the patent term extension originally intended to extend the term of the ‘390 Patent into 2027 will extend the term of the ‘286 Patent into 2027. Similarly, in connection with the conditional approval of Ocaliva for PBC in the European Union, we applied for supplementary patent certification (“SPC”) to extend the patent term for the European analogue of the ‘390 Patent (now the ‘286 Patent) in most of the European Union into 2027. To date, we have received grants of SPC in Austria, Cyprus, Denmark, Finland, France, Germany, Greece, Ireland, Italy, Norway, Portugal, Spain and Sweden. We have also taken similar actions in other jurisdictions and countries where regulations providing for patent term extension exist. The issued patents for OCA are expected to expire between 2022 and 2036 if the appropriate maintenance, renewal, annuity, or other government fees are paid. The substitution in the U.S. of the ‘286 Patent for the ‘390 Patent has no effect on foreign patent rights.

***We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights, and such litigation may divert the attention of our management and scientific personnel and adversely affect our development and commercialization efforts.***

If we choose to file patent infringement lawsuits or engage in other adversarial proceedings to stop another party from making, using, selling, offering for sale or importing the inventions claimed in any of our patents, that individual or company alleged to be infringing has the right to ask the court or adjudicating body to rule that such patents are invalid, not infringed or should not be enforced against that third party. These lawsuits and proceedings are expensive, consume time and resources and divert the attention of management and scientific personnel even if we are successful in defending our rights. In addition, there is a risk that such court or adjudicating body will decide that such patents are invalid, unenforceable or not infringed, and that we do not have the right to stop the other party from making, using, selling, offering for sale or importing the inventions. For example, we have received paragraph IV certification notice letters from several generic drug manufacturers indicating that each such company has submitted to the FDA an ANDA seeking approval to manufacture and sell a generic version of our 5 mg and 10 mg dosage strengths of Ocaliva (obeticholic acid) for PBC prior to the expiration of certain patents protecting Ocaliva. We have initiated patent infringement suits against each of these generic drug manufacturers in the United States District Court for the District of Delaware. Such lawsuits may be expensive and divert our management’s time and attention. In addition, to the extent such lawsuits are not successful, and a generic equivalent of Ocaliva is approved and enters the market before the expiration of our patents protecting Ocaliva, our business may be materially and adversely affected. See Note 18 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q for more information.

Over the past 20 years, the U.S. Supreme Court and the U.S. Congress have modified certain examination procedures utilized by the USPTO in granting patents, which has raised the standard of patentability for some types of inventions. Such modifications may reduce the likelihood that we will be able to obtain patent protection and increase the likelihood of challenges to our patents or the patents we license.

***We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and/or delay, halt or increase the costs of our commercialization efforts.***

Our success will depend in part on our ability to operate without infringing the proprietary rights of third parties. We cannot guarantee that the use, manufacture, sale, offer for sale or importation of our products will not infringe third-party patents. Furthermore, a third party may claim that we or our manufacturing or commercialization partners are using inventions covered by the third party's patent rights and may go to court to stop us from engaging in our normal operations and activities, including making or selling our products and product candidates. The defense of these lawsuits is often costly and could affect our results of operations and divert the attention of our management and scientific personnel. There is also a risk that a court could decide that we or our manufacturing or commercialization partners are infringing the third party's patents and order us or our partners to stop the activities covered by the patents. In that event, we or our partners may be required to halt or delay commercialization or development of the relevant product or product candidate. In addition, there is a risk that a court could order us or our partners to pay the other party damages for having violated the other party's patents, and we may be subject to indemnification obligations with respect to any such payments made by our partners. There is a vast array of patents and patent applications that claim various pharmaceutical inventions and it is not always clear to industry participants, including us, which patents cover various types of products, product candidates or methods of use. The coverage of patents is subject to interpretation by the courts, and such interpretation is not always uniform.

If we are sued for patent infringement, we would need to demonstrate that the relevant patent is not enforceable or that our products, product candidates or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid. Proving invalidity, non-infringement and/or unenforceability is difficult, and we may not be successful. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in such proceedings, we may incur substantial costs and divert our management's time and attention, which could have a material adverse effect on our business. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, which may not be available, defend an infringement action or challenge the validity of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we fail to obtain a license, develop or obtain non-infringing technology or defend an infringement action successfully, we may incur substantial monetary damages, encounter significant delays in the commercialization of our products and product candidates and be precluded from manufacturing or selling our products and product candidates.

We cannot be certain that others have not filed patent applications for technology covered by our pending applications, or that we were the first to invent or file with respect to a technology, because:

- some patent applications in the United States may be unpublished or otherwise maintained in secrecy until the patents are issued;
- patent applications in the United States are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference, derivation or other similar proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful, resulting in a loss of our U.S. patent position with respect to such inventions.

Other countries have similar laws that permit secrecy of patent applications, and such patent applications may be entitled to priority over our applications in such jurisdictions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater financial and other resources. In addition, uncertainties resulting from the initiation and continuation of any such litigation could have a material adverse effect on the market price of our securities and our ability to raise the funds necessary to continue our operations.

***Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated as a result of non-compliance with these requirements.***

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on our patents and patent applications are required to be paid to the USPTO and foreign patent offices in several stages over the lifetime of such patents and patent applications. In addition, the USPTO and foreign patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We have implemented systems and engaged reputable third-party service providers to help ensure that we comply with such requirements on a timely basis, but inadvertent lapses may occur and there are situations in which noncompliance can result in abandonment or lapse of the relevant patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Any such event may impair our competitive position in the relevant jurisdiction and have a material adverse effect on our financial condition or results of operations.

***We may be subject to claims that our employees have wrongfully used or disclosed alleged trade secrets or other proprietary information of their former employers. In addition, if we are not able to adequately prevent disclosure of our trade secrets and other proprietary information, the value of our technology, products and product candidates could be significantly diminished.***

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. We may be subject to claims that these employees, or we, have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims, which could result in substantial costs and be a distraction to our management even if we are successful.

We may rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect, and may not prevent others from independently and lawfully developing similar or identical products that circumvent our intellectual property. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to protect our trade secrets and other proprietary information. These agreements may not effectively prevent disclosure of proprietary information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information.

Third parties, including competitors of ours, may also independently discover our trade secrets or other proprietary information. In addition, we may be required under U.S. or foreign transparency initiatives or other regulations to publicly disclose or otherwise make available certain information that we consider to be proprietary, including pre-clinical and clinical research data. Enforcing a claim that a third party illegally obtained and is using any of our trade secrets or other proprietary information is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside of the United States are sometimes reluctant to protect trade secrets. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain protection of our trade secrets and other proprietary information could adversely affect our competitive business position.

***We have not yet registered all of our trademarks and failure to secure such registrations could adversely affect our business.***

We have numerous trademark and service mark registrations and pending trademark and service mark applications in the United States and abroad.

Our trademark applications may not be allowed for registration and our registered trademarks may not be maintained or enforced. During prosecution of applications for trademark registration, we may receive rejections or refusals. Although we are given an opportunity to respond, we may be unable to overcome such rejections. In addition, the USPTO and comparable agencies in many other jurisdictions provide third parties with an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings have been filed and may in the future be filed against certain of our trademarks, and our trademarks may not survive such proceedings. If we do not secure registrations for our trademarks, we may encounter more difficulty in enforcing them against third parties than we otherwise would.

Trademark protection varies in accordance with local laws. Trademarks remain in force in some countries as long as the trademark is used and in other countries as long as the trademark is registered. Trademark registrations generally are for fixed but renewable terms. We cannot provide any assurances that any trademarks or service marks will be sufficient to prevent competitors from adopting similar names. The adoption of similar names by competitors could impede our ability to build brand identity and may lead to customer confusion, which could adversely affect our sales or profitability.

#### **Risks Related to Our Indebtedness**

***Servicing our debt will require significant amounts of cash, and we may not have sufficient cash flow from our business to effectively service our debt.***

Our ability to make scheduled payments of the principal of, to pay interest on or to refinance the \$460.0 million aggregate principal amount of 2023 Convertible Notes that we issued in July 2016 and/or the \$230.0 million aggregate principal amount of 2026 Convertible Notes that we issued in May 2019 or any other indebtedness we or our subsidiaries may incur in the future depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate cash flow from operations in the future sufficient to service our debt, including the Convertible Notes. If we are unable to generate cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be unfavorable to us or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at the time we seek to refinance such indebtedness. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

***We may incur substantially more debt or take other actions that would affect our ability to pay the principal of and interest on our debt.***

We and our subsidiaries may be able to incur substantial additional debt in the future, some of which may be secured debt. We and our subsidiaries are not restricted under the terms of the indentures governing the Convertible Notes or otherwise from incurring additional debt, securing existing or future debt, recapitalizing our debt or taking other actions that could have the effect of diminishing our ability to service our debt when due.

***The accounting method for convertible debt securities that may be settled in cash, such as the Convertible Notes, could have a material effect on our reported financial results.***

Under Accounting Standards Codification Subtopic 470-20, “Debt with Conversion and Other Options” (“ASC 470-20”), an entity must separately account for the liability and equity components of convertible debt instruments (such as the Convertible Notes) that may be settled entirely or partially in cash upon conversion in a manner that reflects the issuer’s economic interest cost. The effect of ASC 470-20 on the accounting for the Convertible Notes is that the equity component is required to be included in the additional paid-in capital section of stockholders’ equity on our consolidated balance sheets, and the value of the equity component is treated as original issue discount for purposes of accounting for the debt

component of the Convertible Notes. As a result, we are required to record a greater amount of non-cash interest expense in current periods presented as a result of the amortization of the discounted carrying value of the Convertible Notes to their face amount over the term of the Convertible Notes. Because ASC 470-20 requires interest to include both the current period's amortization of the debt discount and the instrument's coupon interest, we report lower net income in our financial results, which could adversely affect the market price of our common stock and the market price of the Convertible Notes.

In addition, under certain circumstances, convertible debt instruments (such as the Convertible Notes) that may be settled entirely or partly in cash are currently accounted for utilizing the treasury stock method, the effect of which is that the shares issuable upon conversion of the Convertible Notes will not be included in the calculation of diluted earnings per share except to the extent that the conversion value of the notes exceeds their principal amount. Under the treasury stock method, for diluted earnings per share purposes, the transaction is accounted for as if the number of shares of common stock that would be necessary to settle such excess, if we elected to settle such excess in shares, are issued. We cannot be sure that the accounting standards in the future will continue to permit the use of the treasury stock method. If we are unable to use the treasury stock method in accounting for the shares issuable upon conversion of the Convertible Notes, then our diluted earnings per share would be adversely affected.

### **Risks Related to Ownership of Our Common Stock**

***Ownership in our common stock is highly concentrated and your ability to influence corporate matters may be limited as a result.***

Our executive officers, directors and stockholders who own more than 5% of our outstanding common stock together beneficially own a significant percentage of our common stock based on reports filed with the SEC. If these stockholders were to choose to act together, they would be able to significantly influence matters submitted to our stockholders for approval, including the election of directors and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization, as well as our management and affairs. This concentration of voting power could delay or prevent an acquisition of us on terms that other securityholders may desire. The interests of this group of stockholders may not always coincide with your interests or the interests of other securityholders and they may act in a manner that advances their best interests and not necessarily those of other securityholders, including seeking a premium value for their common stock, and might affect the market price of our common stock and the Convertible Notes.

***We have a significant stockholder, which will limit your ability to influence corporate matters, may give rise to conflicts of interest and could result in future substantial sales of shares of our common stock into the market.***

Genextra S.p.A. ("Genextra") is one of our largest stockholders and owns a significant minority percentage of our outstanding common stock. Accordingly, Genextra exerts and will continue to exert significant influence over us and any action requiring the approval of the holders of our common stock, including the election of directors and amendments to our organizational documents, such as increases in our authorized shares of common stock and approval of significant corporate transactions. This concentration of voting power makes it less likely that any other holder of common stock will be able to affect the way we are managed and could delay or prevent an acquisition of us on terms that other securityholders may desire.

Furthermore, the interests of Genextra may not always coincide with the interests of other securityholders, and Genextra may act in a manner that advances its best interests and not necessarily those of other securityholders, including seeking a premium value for its common stock, and might affect the market price of our common stock and the Convertible Notes. Our board of directors, which consists of ten directors, including one associated with Genextra, has the power to set the number of directors on our board from time to time.

Genextra also may sell shares of our common stock into the market from time to time, and we cannot predict the effect, if any, that future sales by Genextra may have on the market price of our common stock or the Convertible Notes.

***An active trading market in our common stock may not be maintained.***

The trading market in our common stock has been extremely volatile. The quotation of our common stock on the Nasdaq Global Select Market does not assure that a meaningful, consistent and liquid trading market will exist. We cannot predict whether an active market for our common stock will be maintained in the future. An absence of an active trading market could adversely affect your ability to sell our common stock at current market prices in short time periods, or possibly at all. Additionally, market visibility for our common stock may be limited and such lack of visibility may have a depressive effect on the market price for our common stock.

***We have previously been, and are currently, subject to securities class action litigation and may be subject to similar or other litigation in the future. Such matters can be expensive, time-consuming and have a material adverse effect on our business, results of operations and financial condition.***

We have previously been subject to securities class action lawsuits. In February 2014, two purported securities class actions were filed against us and certain of our officers, which were eventually consolidated. In May 2016, the defendants reached an agreement with the lead plaintiff to seek court approval of a proposed resolution and the settlement was ultimately granted final approval by the court in September 2016. While the final judgment and order of the court included a dismissal of the action with prejudice against all defendants and the defendants did not admit any liability as part of the settlement, the total payment aggregated to \$55.0 million, of which \$10.0 million was paid by our insurers.

In September 2017, a lawsuit and, in January 2018, a follow-on lawsuit, were filed alleging that we and certain of our officers made material misrepresentations and/or omissions of material fact regarding Ocaliva dosing, use and pharmacovigilance-related matters, as well as our operations, financial performance and prospects. The plaintiffs seek unspecified monetary damages on behalf of the putative class, an award of costs and expenses, including attorney's fees, and rescissory damages.

While we believe that we have a number of valid defenses to the claims described above and intend to vigorously defend ourselves, the matters are in the early stages of litigation and no assessment can be made as to the likely outcome of the matters or whether they will be material to us.

Additionally, in November 2020, a lawsuit was filed alleging that we and certain of our officers made material misrepresentations and/or omissions of material fact during the period from September 28, 2019 to October 7, 2020 relating to our NDA for OCA for the treatment of liver fibrosis due to NASH and the use of Ocaliva in patients with PBC, as well as our operations, financial performance and prospects. The plaintiff seeks unspecified monetary damages on behalf of the putative class, and an award of costs and expenses, including attorney's fees.

We may be subject to additional suits or proceedings brought in the future and, as has been the case with many companies in our industry, we may from time to time receive inquiries and subpoenas and other types of information requests from government authorities and others. While the ultimate outcome of any such investigations, inquiries, information requests and legal proceedings is difficult to predict, adverse resolutions or settlements of those matters may result in, among other things, modification of our business practices, product recalls, significant costs, payments, damages or fines or other administrative, civil or criminal remedies, liabilities or penalties, which may have a material adverse effect on our business, results of operations and financial condition. In addition, monitoring and defending against legal actions, whether or not meritorious, and responding to investigations, inquiries and information requests is expensive, time-consuming for our management and detracts from our ability to fully focus our internal resources on our business activities, and we cannot predict how long it may take to resolve such matters. Although we may receive insurance coverage for certain adversarial proceedings, coverage could be denied or prove to be insufficient. It is possible that we could, in the future, incur a judgment or enter into settlement of claims for monetary damages. A decision adverse to our interests could result in the payment of substantial damages and could have a material adverse effect on our business, results of operations and financial condition.

***Our stock price has been and may in the future be volatile, which could cause holders of our common stock to incur substantial losses.***

The market price of our common stock has been, and is likely to continue to be, highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Since our initial public offering in October 2012, the price of our common stock on the Nasdaq Global Select Market has ranged from \$17.96 per share to \$497.00 per share. In addition to the other factors discussed in this “Risk Factors” section and elsewhere in this Quarterly Report on Form 10-Q and in our other periodic filings with the SEC, including our Annual Report on Form 10-K for the year ended December 31, 2019, the factors that may result in wide fluctuations in the price of our common stock include any:

- delay, failure or receipt of regulatory approval for our product candidates, including OCA for liver fibrosis due to NASH;
- delay, failure or receipt of additional marketing authorizations for Ocaliva or our product candidates, including OCA for liver fibrosis due to NASH, in our target markets;
- failure to successfully commercialize our approved products in the United States, Europe and our other target markets, or our inability to maintain regulatory approval for Ocaliva or our other approved products in such markets;
- clinical trial failure, including any such failure resulting from issues, delays or difficulties in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our clinical trials, such as our NASH and PBC trials;
- the effects of COVID-19 and related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions, business closures and disruptions and other public health safety measures;
- inability to obtain additional funding;
- delay in filing an investigational new drug application, NDA, MAA or comparable submission for any of our product candidates, and any adverse development or perceived adverse development with respect to the regulatory review of any such submission;
- potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other product candidates;
- inability to obtain adequate product supply of Ocaliva, OCA for liver fibrosis due to NASH or any of our other product candidates or the inability to do so at acceptable prices;
- results of clinical trials of our competitors’ products and product candidates;
- regulatory or advisory committee actions or recommendations with respect to our products or product candidates, including Ocaliva or OCA for liver fibrosis due to NASH, or our competitors’ products or product candidates;
- changes in laws or regulations applicable to our products or product candidates;
- failure to meet or exceed financial projections or guidance we may provide to the public;
- failure to meet or exceed the estimates and projections of the investment community;
- actual or anticipated fluctuations in our financial condition and operating results;
- actual or anticipated changes in our growth rate relative to our competitors;

- actual or anticipated fluctuations in our competitors' operating results or changes in their growth rate;
- competition from existing products or new products that may emerge;
- announcements by us, our collaborators or our competitors of significant acquisitions, strategic collaborations, joint ventures, collaborations or capital commitments;
- issuance of new or updated research or reports by securities analysts;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- additions or departures of key management or scientific personnel;
- disputes or other developments related to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- announcement or expectation of additional financing efforts;
- disputes, governmental inquiries or investigations, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation;
- sales of our common stock by us, our insiders or our other stockholders;
- failure to adopt appropriate information security systems, including any systems that may be required to support our growing and changing business requirements, or prevent system failures, data breaches or violations of data protection laws;
- market conditions for biopharmaceutical stocks in general; and
- general economic, industry, market and political conditions

Any of these factors could also affect the trading price of the Convertible Notes.

Furthermore, stock markets in general and the market for biotechnology companies in particular have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of securities of many companies. These fluctuations often have been unrelated or disproportionate to the operating performance of those companies. A number of factors, including global health emergencies (e.g., COVID-19), general economic, political and market conditions, recessions, interest rate changes or international currency fluctuations may negatively impact the market price of our securities, regardless of our actual operating performance. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We have been in the past, and are currently subject to this type of litigation, which could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business. As a result of this volatility, you could incur substantial losses.

***If our stockholders sell substantial amounts of our common stock, the market price of our common stock or the Convertible Notes may decline even if our business is doing well.***

A significant number of shares of our common stock are held by a small number of stockholders, including Genextra. Sales of a significant number of shares of our common stock, or the expectation that such sales may occur, could significantly reduce the market price of our common stock or the Convertible Notes. These sales, or the possibility that these sales may occur, also might make it more difficult for us to sell equity securities in the future at a time and price that we deem appropriate. We have also registered the offer and sale of the shares of common stock that we may issue under our equity compensation plans, including upon the exercise of stock options. These shares may be freely sold in the public market upon issuance.

Additionally, sales of our common stock by our executive officers or directors, even when done during an open trading window under our policies with respect to insider sales or done under a trading plan adopted in accordance with the guidelines set forth by Rule 10b5-1, may adversely impact the market price of our common stock or the Convertible Notes. Although we do not expect that the relatively small volume of such sales would itself significantly impact the market price of our common stock or the Convertible Notes, the market could react negatively to the announcement of such sales, which could in turn affect the market price of our common stock and the Convertible Notes.

***You may experience future dilution as a result of future equity offerings or strategic transactions.***

We may in the future raise funds through the issuance and sale of additional shares of our common stock or other securities convertible into or exchangeable for our common stock. For example, in May 2019, we issued and sold an aggregate of 2,879,760 shares of common stock and \$230.0 million aggregate principal amount of the 2026 Convertible Notes, in April 2018, we issued and sold an aggregate of 4,257,813 shares of common stock and in July 2016, we issued and sold \$460.0 million aggregate principal amount of the 2023 Convertible Notes. Conversions of the Convertible Notes will dilute the ownership interests of existing shareholders to the extent that we elect to deliver shares of our common stock (or a combination of cash and shares of our common stock) in connection therewith. In addition, the existence of the Convertible Notes may encourage short selling by market participants because the conversion of the Convertible Notes could depress the price of our common stock. We may also issue shares of common stock, stock options, restricted stock, restricted stock units or other stock-based awards under our existing or future equity incentive plans or other employee or director compensation plans. The issuance of additional shares of common stock (including pursuant to conversions of the Convertible Notes) or other securities convertible into or exchangeable for our common stock, or the perception that such issuances may occur, may materially and adversely affect the price of our common stock and the Convertible Notes.

***Anti-takeover provisions in our restated certificate of incorporation and our restated bylaws, as well as provisions of Delaware law and certain provisions of the Convertible Notes, might discourage, delay or prevent a change in control of our company or changes in our management and, therefore, depress the market price of our common stock or the Convertible Notes.***

Provisions in our restated certificate of incorporation and restated bylaws, as well as provisions of Delaware law, may discourage, delay or prevent a merger, acquisition or other change in control that our securityholders consider favorable, including transactions in which securityholders might otherwise receive a premium for their securities. These provisions may also prevent or frustrate attempts by our stockholders to replace or remove our management. Our corporate governance documents include provisions:

- authorizing the issuance of “blank check” convertible preferred stock, the terms of which may be established and shares of which may be issued without stockholder approval;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders, to the extent that no stockholder, together with its affiliates, holds more than 50% of our voting stock;
- eliminating the ability of stockholders to call a special meeting of stockholders;
- permitting our board of directors to accelerate the vesting of outstanding equity awards upon certain transactions that result in a change of control; and
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings.

In addition, as a Delaware corporation, we are subject to provisions of Delaware law, including Section 203 of the Delaware General Corporation Law (the “DGCL”), which prevents some stockholders holding more than 15% of our outstanding common stock from engaging in certain business combinations without approval of the holders of substantially all of our outstanding common stock. Any provision of our restated certificate of incorporation or restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our

securityholders to receive a premium for their securities, and could also affect the price that some investors are willing to pay for our common stock or the Convertible Notes.

Certain provisions of the Convertible Notes could also make it more difficult or more expensive for a third party to acquire us. For example, if an acquisition event constitutes a “fundamental change” under the terms of the Convertible Notes, holders of the Convertible Notes will have the right to require us to purchase their Convertible Notes for cash. Similarly, if an acquisition event constitutes a “make-whole fundamental change” under the terms of the Convertible Notes, we may be required to increase the conversion rate for holders who convert their Convertible Notes in connection with such make-whole fundamental change.

The existence of the foregoing provisions and anti-takeover measures may also frustrate or prevent any attempts by our stockholders to replace or remove our current management or members of our board of directors and could limit the price that investors might be willing to pay in the future for shares of our common stock or the Convertible Notes. They could also deter potential acquirers of our company, thereby reducing the likelihood that our securityholders could receive a premium for their securities in an acquisition.

***Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful stockholder claims against us and may reduce the amount of money available to us.***

As permitted by Section 102(b)(7) of the DGCL, our restated certificate of incorporation limits the liability of our directors to the fullest extent permitted by law. In addition, as permitted by Section 145 of the DGCL, our restated certificate of incorporation and restated bylaws provide that we shall indemnify, to the fullest extent authorized by the DGCL, each person who is involved in any litigation or other proceeding because such person is or was a director or officer of our company, or is or was serving as an officer or director of another entity at our request, against all expense, loss or liability reasonably incurred or suffered in connection therewith. Our restated certificate of incorporation provides that the right to indemnification includes the right to be paid expenses incurred in defending any proceeding in advance of its final disposition, subject to certain conditions. The rights conferred in the restated certificate of incorporation and the restated bylaws are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons.

The above limitations on liability and our indemnification obligations limit the personal liability of our directors and officers for monetary damages for breach of their fiduciary duty by shifting the burden of such losses and expenses to us. Although we carry directors’ and officers’ liability insurance, certain liabilities or expenses covered by our indemnification obligations may not be covered by such insurance or the coverage limitation amounts may be exceeded. As a result, we may need to use a significant amount of our funds to satisfy our indemnification obligations, which could severely harm our business and financial condition and limit the funds available to securityholders who may choose to bring a claim against our company.

***We do not intend to pay dividends in the foreseeable future.***

We do not anticipate paying cash dividends in the future. As a result, only appreciation of the price of shares of our common stock will provide a return to stockholders, which may not occur. Investors seeking cash dividends should not invest in our common stock. You may not realize any return on your investment in our common stock and may lose some or all of your investment.

***Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.***

We have significant net operating loss carryforwards (“NOLs”) for U.S. Federal, state and foreign income tax purposes. The enactment of the Tax Cuts and Jobs Act enacted in 2017 (the “TCJA”) modified the ability of companies to utilize U.S. Federal NOLs arising in tax years beginning on or after January 1, 2018, by providing that such NOLs may be carried-forward indefinitely and used to offset up to 80 percent of taxable income in any given future year. Existing NOLs that arose in tax years beginning prior to January 1, 2018, were not affected by the TCJA and are generally eligible to be carried-forward for up to 20 years and used to fully offset taxable income in future years. If not utilized, our pre-2018

NOLs will expire for U.S. Federal income tax purposes between 2024 and 2037. We also have certain state and foreign NOLs in varying amounts depending on the different state and foreign tax laws.

In addition, our ability to utilize our NOLs may be limited under Section 382 of the Internal Revenue Code or applicable state and foreign tax law. The Section 382 limitations apply if an “ownership change” occurs. Generally, an ownership change occurs when certain shareholders increase their aggregate ownership by more than 50 percentage points over their lowest ownership percentage in a testing period (typically three years). We have evaluated whether one or more ownership changes under Section 382 have occurred since our inception and have determined that there have been at least two such changes. Although we believe that these ownership changes have not resulted in material limitations on our ability to use these NOLs, our ability to utilize these NOLs may be limited due to future ownership changes or for other reasons. As a result, we may not be able to take full advantage of our NOL carryforwards for U.S. Federal, state, and foreign income tax purposes.

### General Risk Factors

***We may use our limited financial and human resources to pursue a particular research program or product candidate that is ultimately unsuccessful or less successful than other programs or product candidates that we may have forgone or delayed.***

Because we have limited resources, we may forego or delay the development of certain programs or product candidates that later prove to have greater commercial potential than the programs or product candidates that we do pursue. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we fail to accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other arrangements or we may allocate our limited internal resources to that product candidate when it would have been more advantageous to enter into such an arrangement. Any such failure could have a material adverse effect on our business, financial condition or results of operations.

***If we engage in a licensing transaction, acquisition, reorganization or business combination, we will face a variety of risks that could adversely affect our business operations and our securityholders.***

From time to time, we have considered, and we will continue to consider in the future, strategic business initiatives intended to further the expansion and development of our business. These initiatives may include in-licensing or acquiring products, technologies or businesses, entering into a business combination with another company or otherwise partnering with another company. If we pursue such a strategy, we could, among other things:

- issue equity securities that would dilute our current stockholders’ ownership;
- incur substantial debt that may place strains on our operations;
- be required to dedicate substantial operational, financial and management resources to integrate new products, technologies or businesses;
- assume substantial actual or contingent liabilities;
- impair our ability to make payments of interest and principal on our outstanding debt, including the Convertible Notes;
- reprioritize our development programs or cease development and commercialization activities with respect to certain of our product candidates or approved products; or

- merge or otherwise enter into a business combination with another company, which may result in our stockholders receiving cash and/or securities of the other company on terms that certain of our stockholders may not deem desirable.

***Our insurance policies are expensive and only protect us from some business risks, which leave us exposed to significant uninsured liabilities.***

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, employment practices liability, property, auto, workers' compensation, cyber liability, products liability and directors' and officers' insurance. We do not know, however, if our current levels of coverage are adequate or if we will be able to obtain insurance with adequate levels of coverage in the future, if at all. Any significant uninsured liability, including significant uninsured liabilities resulting from COVID-19 or related government-imposed shelter-in-place mandates, restrictions on travel and in-person interactions, business closures and disruptions, and other public health safety measures, may require us to pay substantial amounts, which could materially and adversely affect our financial position and results of operations. Furthermore, any increase in the volatility of our stock price, among other factors, may result in us being required to pay substantially higher premiums for our directors' and officers' insurance, and may make it difficult for us to obtain adequate coverage on reasonable terms, if at all.

***We must comply with environmental, health and safety laws and regulations.***

Our activities involve the controlled storage, use and disposal of hazardous materials. We are subject to federal, state, city and local laws and regulations, in and outside the United States, governing the use, manufacture, storage, handling and disposal of these hazardous materials. Although we believe that the safety procedures we use for handling and disposing of these materials comply with the standards prescribed by applicable laws and regulations, we cannot eliminate the risk of accidental contamination or injury from these materials. In the event of an accident, regulatory authorities may curtail the use of these materials and interrupt our business operations. We do not currently maintain hazardous materials insurance coverage.

***Failure to establish and maintain adequate financial infrastructure and accounting systems and controls could impair our ability to comply with the financial reporting and internal controls requirements for publicly traded companies.***

As a public company, we operate in a demanding regulatory environment, which requires us to comply with the Sarbanes-Oxley Act of 2002 and related rules and regulations, expanded disclosure requirements, accelerated reporting requirements and complex accounting rules. Responsibilities imposed by the Sarbanes-Oxley Act include establishing and maintaining corporate oversight and adequate internal control over financial reporting and disclosure controls and procedures. Effective internal controls are necessary for us to produce reliable financial reports and are important to help prevent financial fraud.

In particular, our compliance with Section 404 of the Sarbanes-Oxley Act has required and will continue to require that we incur substantial accounting-related expenses and expend significant management efforts. Our testing, or the testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls that we would be required to remediate in a timely manner. If we are not able to comply with the requirements of the Sarbanes-Oxley Act, we could be subject to sanctions or investigations by the SEC, the Nasdaq Global Select Market or other regulatory authorities, which would require additional financial and management resources and could adversely affect the market price of our securities. Furthermore, if we cannot provide reliable financial reports or prevent fraud, including as a result of remote working by our employees in connection with COVID-19 and related government-imposed shelter-in-place mandates and other public health safety measures, our business and results of operations would likely be materially and adversely affected.

***Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.***

We are subject to the periodic reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within

the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosure due to error or fraud may occur and not be detected.

***Changes in our effective income tax rate could adversely affect our results of operations.***

We are subject to income taxes in the United States and various foreign jurisdictions. Various factors may have favorable or unfavorable effects on our effective income tax rate. These factors include, but are not limited to, interpretations of existing tax laws, changes in tax laws and rates, such as the TCJA, the accounting for stock options and other stock-based compensation, changes in accounting standards, future levels of research and development spending, changes in the mix and level of pre-tax earnings in different jurisdictions, the outcome of audits or other examinations by the U.S. Internal Revenue Service and tax regulators in other jurisdictions, the accuracy of our estimates for unrecognized tax benefits, the realization of deferred tax assets and changes to our ownership or capital structure.

The impact on our effective income tax rate resulting from these factors may be significant and could adversely affect our results of operations.

***If securities or industry analysts cease publishing research or reports about us, our business or our market, or if they publish inaccurate or unfavorable reports about us or our securities, the price of our securities and trading volume in our securities could decline.***

The market for our common stock and the Convertible Notes depends in part on the research and reports that securities or industry analysts publish about our company. We do not have any control over these analysts, and there can be no assurance that analysts will continue to cover us or provide favorable coverage. If one or more of the analysts who cover us downgrade our common stock or publish inaccurate or unfavorable research about our business, our stock price and the price of the Convertible Notes may decline. If one or more of the analysts covering us fail to regularly publish reports on us, demand for our common stock and the Convertible Notes may decline, which could cause our stock price and the price of the Convertible Notes and trading volume to decline.

**Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.**

**Recent Sales of Unregistered Securities**

Not applicable.

**Issuer Purchases of Equity Securities**

The following table provides certain information with respect to purchases of our common stock during the three months ended September 30, 2020.

<u>Period</u>	<u>Total Number of Shares Purchased (1)</u>	<u>Average Price Paid per Share</u>	<u>Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs</u>	<u>Maximum Number (or Approximate Dollar Value) of Shares that May Yet Be Purchased Under the Plans or Programs</u>
July 1, 2020 through July 31, 2020	1,657	\$ 46.52	—	—
August 1, 2020 through August 31, 2020	1,297	\$ 49.82	—	—
September 1, 2020 through September 30, 2020	69	\$ 43.41	—	—
Total	<u>3,023</u>	<u>\$ 47.86</u>	<u>—</u>	<u>—</u>

(1) Represents shares of common stock withheld to satisfy taxes associated with the vesting of restricted stock awards.

**Item 6. Exhibits.**

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

**Exhibit Index**

<b>Exhibit Number</b>	<b>Description of Exhibit</b>
31.1	<a href="#">Certification of Principal Executive Officer required by Rule 13a-14(a) or Rule 15d-14(a).</a>
31.2	<a href="#">Certification of Principal Financial Officer required by Rule 13a-14(a) or Rule 15d-14(a).</a>
32.1(1)	<a href="#">Certifications 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).</a>
101	The following materials from the Registrant’s Quarterly Report on Form 10-Q for the period ended September 30, 2020, formatted in Inline XBRL (Inline eXtensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets at September 30, 2020 (unaudited) and December 31, 2019 (audited), (ii) Condensed Consolidated Statements of Operations for the three and nine-month periods ended September 30, 2020 and 2019 (unaudited), (iii) Condensed Consolidated Statements of Comprehensive Loss for the three and nine-month periods ended September 30, 2020 and 2019 (unaudited), (iv) Condensed Consolidated Statements of Changes in Stockholders’ (Deficit) Equity for the three and nine-month periods ended September 30, 2020 and 2019 (unaudited), (v) Condensed Consolidated Statements of Cash Flows for the nine-month periods ended September 30, 2020 and 2019 (unaudited) and (vi) Notes to Condensed Consolidated Financial Statements (unaudited).
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).

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- (1) The certifications attached hereto as Exhibit 32.1 are furnished to the SEC pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, nor shall they be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, except as shall be expressly set forth by specific reference in such filing.

**SIGNATURES**

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

**INTERCEPT PHARMACEUTICALS, INC.**

Date: November 9, 2020

By: /s/ Mark Pruzanski, M.D.  
Mark Pruzanski, M.D.  
President and Chief Executive Officer  
(Principal Executive Officer)

Date: November 9, 2020

By: /s/ Sandip Kapadia  
Sandip Kapadia  
Chief Financial Officer and Treasurer  
(Principal Financial Officer)

## CERTIFICATION

I, Mark Pruzanski, M.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Intercept Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent 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 9, 2020

By: /s/ Mark Pruzanski, M.D.  
Mark Pruzanski, M.D.  
President and Chief Executive Officer  
(Principal Executive Officer)

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## CERTIFICATION

I, Sandip Kapadia, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Intercept Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent 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 9, 2020

By: /s/ Sandip Kapadia  
Sandip Kapadia  
Chief Financial Officer and Treasurer  
(Principal Financial Officer)

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## 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), Mark Pruzanski, M.D., President and Chief Executive Officer of Intercept Pharmaceuticals, Inc. (the "Company"), and Sandip Kapadia, Chief Financial Officer and Treasurer of the Company, each hereby certifies that, to the best of his knowledge:

(1) The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2020, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

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

Date: November 9, 2020

By: /s/ Mark Pruzanski, M.D.  
Mark Pruzanski, M.D.  
President and Chief Executive Officer  
(Principal Executive Officer)

Date: November 9, 2020

By: /s/ Sandip Kapadia  
Sandip Kapadia  
Chief Financial Officer and Treasurer  
(Principal Financial Officer)

A signed original of this written statement required by Rule 13a-14(b) of the Exchange Act and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350) has been provided to Intercept Pharmaceuticals, Inc. and will be retained by Intercept Pharmaceuticals, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies the Quarterly Report on 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 Intercept Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Quarterly Report on Form 10-Q), irrespective of any general incorporation language contained in such filing.

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