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, 2019

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	**22-3868459**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)

10 Hudson Yards, 37th 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:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
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 ☒	Accelerated filer ☐
Non-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, 2019 was 32,733,447.

Intercept Pharmaceuticals, Inc.

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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 development candidates, including OCA for NASH, the timing and acceptance of our potential regulatory filings and potential approval of OCA for NASH or any other indications in addition to PBC, the timing and potential commercial success of OCA and any other product candidates we may develop and our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans, objectives of management and expected market growth.

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;
- 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 timely and cost-effectively file for and obtain regulatory approval of our product candidates, including OCA for NASH, in the United States, Europe and our other target markets;
- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), 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 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;
- 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 NASH, and our clinical trial activities;
- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to timely and successfully launch OCA for 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 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 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, 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 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, 2018.

NOTE REGARDING TRADEMARKS

The Intercept Pharmaceuticals® name and logo and the Ocaliva® 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 ® and ™ 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.

PART I

Item 1. Financial Statements.

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

	September 30, 2019 (Unaudited)	December 31, 2018 (Audited)
Assets		
Current assets:		
Cash and cash equivalents	$ 63,377	$ 43,248
Investment debt securities, available-for-sale	648,994	392,912
Accounts receivable, net	32,822	25,694
Prepaid expenses and other current assets	23,891	20,571
Total current assets	769,084	482,425
Fixed assets, net	5,973	10,411
Inventory, net	9,348	7,108
Security deposits	8,205	9,223
Other assets	9,484	—
Total assets	$ 802,094	$ 509,167
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses and other liabilities	$ 133,295	$ 105,109
Short-term interest payable	5,463	7,475
Short-term portion of deferred revenue	1,216	1,621
Total current liabilities	139,974	114,205
Long-term liabilities:		
Long-term debt	525,339	371,250
Long-term other liabilities	6,046	3,771
Long-term portion of deferred revenue	—	811
Total liabilities	$ 671,359	$ 490,037
Commitments and contingencies (Note 15)		
Stockholders' equity:		
Common stock par value $0.001 per share; 45,000,000 shares authorized; 32,733,447 and 29,693,876 shares issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	33	30
Additional paid-in capital	2,157,080	1,800,144
Accumulated other comprehensive loss, net	(1,070)	(2,259)
Accumulated deficit	(2,025,308)	(1,778,785)
Total stockholders' equity	130,735	19,130
Total liabilities and stockholders' equity	$ 802,094	$ 509,167

See accompanying notes to the condensed consolidated financial statements.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Revenue:				
Product revenue, net	$ 61,545	$ 46,581	$ 179,286	$ 124,908
Licensing revenue	405	405	1,216	1,616
Total revenue	61,950	46,986	180,502	126,524
Operating expenses:				
Cost of sales	487	519	1,738	1,512
Selling, general and administrative	76,828	56,812	223,738	184,503
Research and development	60,168	47,941	178,163	144,028
Total operating expenses	137,483	105,272	403,639	330,043
Operating loss	(75,533)	(58,286)	(223,137)	(203,519)
Other income (expense):				
Interest expense	(11,795)	(7,671)	(29,518)	(22,769)
Other income, net	2,495	1,503	6,132	5,051
	(9,300)	(6,168)	(23,386)	(17,718)
Net loss	$ (84,833)	$ (64,454)	$ (246,523)	$ (221,237)
Net loss per common and potential common share:				
Basic and diluted	$ (2.59)	$ (2.18)	$ (7.88)	$ (7.89)
Weighted average common and potential common shares outstanding:				
Basic and diluted	32,717	29,615	31,275	28,057

See accompanying notes to the condensed consolidated financial statements.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Net loss	$ (84,833)	$ (64,454)	$ (246,523)	$ (221,237)
Other comprehensive (loss) income:				
Net changes related to available-for-sale investment debt securities:				
Unrealized gains on investment debt securities	451	368	1,646	119
Reclassification adjustment for realized gains on investment debt securities included in other income, net	2	—	6	—
Net unrealized gains on investment debt securities	$ 453	$ 368	$ 1,652	$ 119
Foreign currency translation gains (losses)	(687)	63	(463)	(979)
Other comprehensive (loss) income	$ (234)	$ 431	$ 1,189	$ (860)
Comprehensive loss	$ (85,067)	$ (64,023)	$ (245,334)	$ (222,097)

See accompanying notes to the condensed consolidated financial statements.

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

Three months ended September 30, 2019

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity
	Shares	Amount				
Balance - June 30, 2019	32,696	$ 33	$ 2,116,481	$ (836)	$ (1,940,475)	$ 175,203
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	32,733	$ 33	$ 2,157,080	$ (1,070)	$ (2,025,308)	$ 130,735

Nine months ended September 30, 2019

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity
	Shares	Amount				
Balance - December 31, 2018	29,694	$ 30	$ 1,800,144	$ (2,259)	$ (1,778,785)	$ 19,130
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	32,733	$ 33	$ 2,157,080	$ (1,070)	$ (2,025,308)	$ 130,735

Three months ended September 30, 2018

	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity
Balance - June 30, 2018	29,565	$ 29	$ 1,774,955	$ (2,082)	$ (1,626,326)	$ 146,576
Stock-based compensation	—	—	11,994	—	—	11,994
Net proceeds from exercise of stock options	89	1	2,715	—	—	2,716
Employee withholding taxes related to stock-based awards	—	—	(1,459)	—	—	(1,459)
Other comprehensive income	—	—	—	436	—	436
Net loss	—	—	—	—	(64,454)	(64,454)
Balance - September 30, 2018	29,654	$ 30	$ 1,788,205	$ (1,646)	$ (1,690,780)	$ 95,809

Nine months ended September 30, 2018

	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity
Balance - December 31, 2017	25,173	$ 25	$ 1,486,690	$ (786)	$ (1,469,543)	$ 16,386
Stock-based compensation	—	—	38,415	—	—	38,415
Issuance of common stock from public and private placement offerings, net of underwriting fees and issuance costs	4,258	5	261,357	—	—	261,362
Net proceeds from exercise of stock options	223	—	3,201	—	—	3,201
Employee withholding taxes related to stock-based awards	—	—	(1,458)	—	—	(1,458)
Other comprehensive loss	—	—	—	(860)	—	(860)
Net loss	—	—	—	—	(221,237)	(221,237)
Balance - September 30, 2018	29,654	$ 30	$ 1,788,205	$ (1,646)	$ (1,690,780)	$ 95,809

See accompanying notes to the condensed consolidated financial statements.

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

	Nine Months Ended September 30, 2019	2018
Cash flows from operating activities:		
Net loss	$ (246,523)	$ (221,237)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	42,809	38,415
(Accretion) amortization of (discount) premium on investment debt securities	(595)	266
Amortization of deferred financing costs	1,529	1,144
Depreciation	2,839	3,551
Non-cash operating lease cost	3,974	—
Gain on lease termination	(1,995)	—
Loss on the disposal of fixed assets	2,682	1,331
Accretion of debt discount	15,051	10,412
Unrealized gain on investments	—	(119)
Changes in operating assets:		
Prepaid expenses and other current assets	(3,320)	(856)
Accounts receivable	(7,128)	(5,579)
Inventory	(2,240)	(3,995)
Security deposits	1,018	7,138
Other assets	(19,489)	—
Changes in operating liabilities:		
Accounts payable, accrued expenses and other current liabilities	33,990	(11,719)
Operating lease liabilities	(5,019)	—
Long-term other liabilities	9,516	(1,300)
Interest payable	(2,012)	(3,738)
Deferred revenue	(1,216)	(1,616)
Net cash used in operating activities	(176,129)	(187,902)
Cash flows from investing activities:		
Purchases of investment debt securities	(560,733)	(378,261)
Sales and maturities of investment debt securities	306,898	293,307
Purchases of equipment, leasehold improvements, and furniture and fixtures	(1,041)	(45)
Net cash used by investing activities	(254,876)	(84,999)
Cash flows from financing activities:		
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	261,362
Proceeds from exercise of options, net	2,511	3,201
Payments of employee withholding taxes related to stock-based awards	(1,476)	(1,458)
Net cash provided by financing activities	451,639	263,105
Effect of exchange rate changes	(505)	(835)
Net increase/(decrease) in cash and cash equivalents	20,129	(10,631)
Cash and cash equivalents – beginning of period	43,248	70,013
Cash and cash equivalents – end of period	$ 63,377	$ 59,382

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 Securities and Exchange Commission ("SEC"). Operating results for the three and nine months ended September 30, 2019 are not necessarily indicative of the results that may be expected for any future period or for the year ending December 31, 2019. 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, 2018, included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018 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.

3. Summary of Significant Accounting Policies

The Company's significant accounting policies are described in Note 2 of Notes to Consolidated Financial Statements included in its Annual Report on Form 10-K for the year ended December 31, 2018 filed with the SEC. Other than the adoption of Accounting Standards Codification ("ASC") Topic 842, *Leases* ("ASC 842") as of January 1, 2019, as described below, there were no other significant changes to the Company's significant accounting policies.

Leases

The Company determines if an arrangement is a lease at inception and records right-of-use ("ROU") assets and lease liabilities on the condensed consolidated balance sheets at lease commencement based on the present value of remaining lease payments over the lease term. The Company only considers payments that are fixed and determinable at the time of commencement. Operating leases are included in other assets, accounts payable, accrued expenses and other liabilities and long-term other liabilities on the condensed consolidated balance sheets.

Operating lease liabilities are recognized based on the present value of the future minimum lease payments discounted by the Company's incremental borrowing rate. The Company measures ROU assets based on the corresponding lease liability adjusted for (i) payments made to the lessor at or before the commencement date, (ii) initial direct costs incurred and (iii) tenant incentives under the lease. The Company's lease terms may include options to extend or terminate the lease

when it is reasonably certain that it will exercise that option. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term.

For short-term leases, the Company records rent expense in its consolidated statements of operations on a straight-line basis over the lease term and records variable lease payments as incurred.

Additional information and disclosures are contained in Note 8 — Operating Leases below.

Recent Accounting Pronouncements

Recently Adopted Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board ("FASB") established ASC 842, by issuing Accounting Standards Update ("ASU") No. 2016-02, which requires lessees to recognize leases on-balance sheet and disclose key information about leasing arrangements. ASC 842 was subsequently amended by ASU No. 2018-01, "Land Easement Practical Expedient for Transition to Topic 842"; ASU No. 2018-10, "Codification Improvements to Topic 842, Leases"; and ASU No. 2018-11, "Targeted Improvements". The new standard establishes a ROU model that requires a lessee to recognize a ROU asset and lease liability on the balance sheet for all leases with a term longer than 12 months. Leases will be classified as finance or operating, with classification affecting the pattern and classification of expense recognition in the income statement. A modified retrospective transition approach is required, applying the new standard to all leases existing at the date of initial application. An entity may choose to use either (i) its effective date or (ii) the beginning of the earliest comparative period presented in the financial statements as its date of initial application. The Company adopted the new standard on January 1, 2019 using the effective date as the date of initial application. The new standard provides a number of optional practical expedients in transition. The Company elected the "package of practical expedients", which permits the Company to not reassess under the new standard the Company's prior conclusions about lease identification, lease classification and initial direct costs. The new standard also provides practical expedients for an entity's ongoing accounting. The Company elected the short-term lease recognition exemption for all leases that qualify. This means, for those leases that qualify, the Company will not recognize ROU assets or lease liabilities, and this includes not recognizing ROU assets or lease liabilities for existing short-term leases of those assets in transition. The Company also elected the practical expedient to not separate lease and non-lease components for all of the Company's leases. Upon adoption, the Company recognized additional operating liabilities of $25.4 million, with corresponding ROU assets of $19.6 million based on the present value of the remaining minimum rental payments under current leasing standards for existing operating leases.

In July 2017, the FASB issued ASU No. 2017-11, "Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480) and Derivatives and Hedging (Topic 815): I. Accounting for Certain Financial Instruments with Down Round Features; II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception" ("ASU 2017-11"). Part I of this update addresses the complexity of accounting for certain financial instruments with down round features. Down round features are features of certain equity-linked instruments (or embedded features) that result in the strike price being reduced on the basis of the pricing of future equity offerings. Current accounting guidance creates cost and complexity for entities that issue financial instruments (such as warrants and convertible instruments) with down round features that require fair value measurement of the entire instrument or conversion option. Part II of this update addresses the difficulty of navigating ASC Topic 480, *Distinguishing Liabilities from Equity*, because of the existence of extensive pending content in the FASB Accounting Standards Codification. This pending content is the result of the indefinite deferral of accounting requirements about mandatorily redeemable financial instruments of certain nonpublic entities and certain mandatorily redeemable noncontrolling interests. The amendments in Part II of this update do not have an accounting effect. This ASU is effective for fiscal years, and interim periods within those years, beginning after December 15, 2018. The Company adopted ASU 2017-11 on January 1, 2019 and its adoption did not have any impact on the Company's consolidated financial statements and related disclosures.

In June 2018, the FASB issued ASU No. 2018-07, "Improvements to Nonemployee Share-Based Payment Accounting" ("ASU 2018-07"), which simplifies the accounting for share-based payments granted to nonemployees for goods and services. Under this ASU, most of the guidance on such payments to nonemployees would be aligned with the

requirements for share-based payments granted to employees. The changes take effect for public companies for fiscal years starting after December 15, 2018, including interim periods within that fiscal year. For all other entities, the amendments are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted, but no earlier than an entity's adoption date of ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"). The Company adopted ASU 2018-07 on January 1, 2019 on a modified retrospective basis through a cumulative-effect adjustment to equity by remeasuring, on that date, the fair value of all outstanding unvested stock options that had been granted to nonemployees. The adoption did not have a material impact on the Company's consolidated financial statements and related disclosures.

Recent Accounting Pronouncements to be Adopted

In June 2016, the FASB issued ASU No. 2016-13, "Financial Instruments-Credit Losses: Measurement of Credit Losses on Financial Instruments" ("ASU 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 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. 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 is currently evaluating the impact of this standard on its 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 plans to adopt ASU 2018-13 effective January 1, 2020 and does not expect the adoption of this guidance to have a material impact on the Company's consolidated financial statements and related disclosures.

4. Significant Agreements

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 May 2014, Sumitomo Dainippon exercised the Country Option in part to add Korea as part of its licensed territories and paid the Company a $1.0 million upfront fee in connection therewith. In February 2018, the Company and Sumitomo Dainippon entered into Amendment No. 3 (the "Sumitomo Amendment") to the Original Sumitomo Agreement (as amended, the "Sumitomo Agreement"). Pursuant to the Sumitomo Amendment, (i) Sumitomo Dainippon agreed to return the rights to develop and commercialize OCA in Japan and Korea and waived its rights to the Country Option, (ii) the Company agreed to forego any further milestone or royalty payments relating to the development and commercialization of OCA in Japan and Korea and (iii) certain milestone payment obligations with respect to the development and commercialization of OCA were adjusted. In addition, the Company and Sumitomo Dainippon agreed that if certain clinical development milestones in China are not met by December 31, 2020, Sumitomo Dainippon may choose either to make a milestone payment to the Company or terminate the Sumitomo Agreement. Sumitomo Dainippon may also terminate the Sumitomo Agreement in its entirety or on an indication-by-indication basis at any time upon 90 days' written notice. As of September 30, 2019, the Company had achieved $6.0

million of development milestones under the Sumitomo Agreement. The Company may be eligible to receive additional milestone payments under the Sumitomo Agreement in an aggregate amount of up to approximately $23.0 million based on the occurrence of certain clinical trial and regulatory-related events and tiered royalty payments up to the mid-twenties in percentage terms based on net sales of OCA products in China (excluding Taiwan). Sumitomo Dainippon is responsible for the costs of developing and commercializing OCA in its territory.

The Company has concluded that Sumitomo Dainippon does not represent a customer of the Company, and therefore the Sumitomo Agreement is outside of the scope of ASC 606. The Company has accounted, and continues to account, for the Sumitomo Agreement under the legacy accounting guidance. The Company's substantive performance obligations under this agreement include an exclusive license to its technology, technical and scientific support to the development plan and participation on a joint steering committee. The Company determined that these performance obligations represent a single unit of accounting, since, initially, the license does not have stand-alone value to Sumitomo Dainippon without the Company's technical expertise and steering committee participation during the development of OCA. The development period is currently estimated as continuing through June 2020 and, as such, the $15.0 million upfront payment is being recognized ratably over this period. The Company recognized licensing revenue of $0.4 million and $0.4 million for the three months ended September 30, 2019 and 2018, respectively, and $1.2 million and $1.6 million for the nine months ended September 30, 2019 and 2018, respectively, under the Sumitomo Agreement. Included in licensing revenue for the nine months ended September 30, 2018 is $0.4 million related to the accelerated recognition, as a result of the Sumitomo Amendment, of the remaining portion of deferred revenue associated with the $1.0 million upfront payment that the Company received under the Original Sumitomo Agreement in connection with Sumitomo Dainippon's exercise of the Country Option with respect to Korea.

The Company recognizes milestone payments when the associated milestones are achieved. As of September 30, 2019, and December 31, 2018, the Company had recorded deferred revenues of $1.2 million and $2.4 million, respectively, under this agreement.

5. 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, 2019 and December 31, 2018:

	As of September 30, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
Cash and cash equivalents:				
Cash and money market funds	$ 63,377	$ —	$ —	$ 63,377
Total cash and cash equivalents	63,377	—	—	63,377
Investment debt securities:				
Commercial paper	70,009	62	(8)	70,063
Corporate debt securities	574,046	984	(95)	574,935
U.S. government and agency securities	3,992	4	—	3,996
Total investment debt securities	648,047	1,050	(103)	648,994
Total cash, cash equivalents and investment debt securities	$ 711,424	$ 1,050	$ (103)	$ 712,371

	As of December 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
Cash and cash equivalents:				
Cash and money market funds	$ 43,248	$ —	$ —	$ 43,248
Investment debt securities:				
Commercial paper	34,353	—	(26)	34,327
Corporate debt securities	349,854	27	(704)	349,177
U.S. government and agency securities	9,410	5	(7)	9,408
Total investment debt securities	393,617	32	(737)	392,912
Total cash, cash equivalents and investment debt securities	$ 436,865	$ 32	$ (737)	$ 436,160

As of September 30, 2019, the Company held a total of five positions that were in a continuous unrealized loss position for twelve months or longer. The Company has determined that the unrealized losses are deemed to be temporary impairments as of September 30, 2019. The Company believes that the unrealized losses generally are caused by increases in the risk premiums required by market participants rather than an adverse change in cash flows or a fundamental weakness in the credit quality of the issuer or underlying assets. Because the Company has the ability and intent to hold these investments until a recovery of fair value, which may be maturity, it does not consider the investments to be other-than-temporarily impaired at September 30, 2019.

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, 2019	December 31, 2018
		(in thousands)	
Office equipment and software	3	$ 4,343	$ 3,986
Leasehold improvements	Over life of lease	10,444	14,464
Furniture and fixtures	7	3,974	3,907
Subtotal		18,761	22,357
Less: accumulated depreciation		(12,788)	(11,946)
Fixed assets, net		$ 5,973	$ 10,411

7. Inventory, Net

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

	September 30, 2019	December 31, 2018
	(in thousands)	
Work-in-process	$ 9,289	$ 7,019
Finished goods	59	89
Inventory, net	$ 9,348	$ 7,108

8. Operating Leases

The Company leases various office spaces under non-cancelable operating leases with original lease periods expiring between the fourth quarter of 2019 and 2024. The Company subleases one of its office spaces to a third party. 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 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 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.

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

Leases	Classification	September 30, 2019
Assets		(in thousands)
Operating lease assets	Other assets	$ 9,484
Total leased assets		$ 9,484
Liabilities		
Current		
Operating lease liabilities	Accounts payable, accrued expenses and other liabilities	$ 6,215
Noncurrent		
Operating lease liabilities	Long-term other liabilities	6,028
Total lease liabilities		$ 12,243

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

Lease Cost	Classification	Three Months Ended September 30, 2019 (in thousands)	Nine Months Ended September 30, 2019 (in thousands)
Operating lease cost	Selling, general and administrative expenses	$ 1,456	$ 4,603
Short-term lease cost	Selling, general and administrative expenses	392	1,746
Variable lease cost	Selling, general and administrative expenses	230	626
Sublease income	Other income, net	(182)	(546)
Net lease cost		$ 1,896	$ 6,429

The weighted-average remaining term of the Company's operating leases was 2.5 years and the weighted-average discount rate used to measure the present value of the Company's operating lease liabilities was 5.0% as of September 30, 2019.

Maturities of the Company's operating lease liabilities, which do not include short-term leases, as of September 30, 2019 are as follows:

Maturity of Lease Liabilities	Operating leases
	(in thousands)
2019	$ 1,889
2020	6,142
2021	2,828
2022	896
2023	896
Thereafter	374
Total lease payments	13,025
Less: Present value discount	(782)
Total operating lease liabilities	$ 12,243

Cash payments included in the measurement of the Company's lease liabilities were $5.5 million for the nine months ended September 30, 2019.

9. Accounts Payable, Accrued Expenses and Other Liabilities

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

	September 30, 2019	December 31, 2018
	(in thousands)	
Accounts payable	$ 19,485	$ 11,765
Accrued employee compensation	19,343	20,335
Accrued contracted services	66,231	54,681
Other liabilities	22,021	18,328
Operating lease liabilities	6,215	—
Accounts payable, accrued expenses and other liabilities	$ 133,295	$ 105,109

Research & Development Tax Credit

The Company benefits from the U.K. Small and Medium-sized Enterprise R&D Tax Credit scheme, or the SME scheme, under which it can obtain a refundable 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 Her Majesty's Revenue and Customs, the U.K.'s government tax authority. Given the claim review has not been finalized, the credit received is recorded as a deferred liability within Accounts payable, accrued expenses, and other liabilities.

10. Fair Value Measurements

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

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 market prices. 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:

		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
		(in thousands)		
September 30, 2019				
Assets				
Cash and cash equivalents:				
Money market funds (included in cash and cash equivalents)	$ 31,691	$ 31,691	$ —	$ —
Available-for-sale debt securities:				
Commercial paper	70,063	—	70,063	—
Corporate debt securities	574,935	—	574,935	—
U.S. government and agency securities	3,996	—	3,996	—
Total financial assets	$ 680,685	$ 31,691	$ 648,994	$ —
December 31, 2018				
Assets				
Money market funds (included in cash and cash equivalents)	$ 11,647	$ 11,647	$ —	$ —
Available-for-sale debt securities:				
Commercial paper	34,327	—	34,327	—
Corporate debt securities	349,177	—	349,177	—
U.S. government and agency securities	9,408	—	9,408	—
Total financial assets	$ 404,559	$ 11,647	$ 392,912	$ —

The estimated fair value of the available-for-sale debt securities (commercial paper, corporate debt securities and U.S. government and agency securities), by contractual maturity, are as follows:

	Fair Value as of	
	September 30, 2019	December 31, 2018
	(in thousands)	
Due in one year or less	$ 486,971	$ 319,717
Due after one year through two years	162,023	73,195
Total investments in debt securities	$ 648,994	$ 392,912

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

11. Long-Term Debt

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

	September 30, 2019	December 31, 2018
	(in thousands)	
2023 Convertible Notes	$ 460,000	$ 460,000
2026 Convertible Notes	230,000	—
Long-term debt, gross	690,000	460,000
Less: Unamortized debt discounts and fees	164,661	88,750
Long-term debt, net	525,339	371,250

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 September 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 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 $213.9 million at September 30, 2019 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 $400.7 million and $410.9 million at September 30, 2019 and December 31, 2018, respectively, and was determined using Level 2 inputs based on quoted market values.

Interest Expense on Convertible Notes

Interest expense was $11.8 million and $7.7 million for the three months ended September 30, 2019 and 2018, respectively, and $29.5 million and $22.8 million for the nine months ended September 30, 2019 and 2018, respectively,

related to the Convertible Notes. Accrued interest on the Convertible Notes was approximately $5.5 million and $7.5 million as of September 30, 2019 and December 31, 2018, respectively. The Company recorded debt issuance costs of $19.0 million, which are being amortized using the effective interest method. As of September 30, 2019, $13.8 million of debt issuance costs are recorded on the unaudited condensed consolidated balance sheet in Long-term debt, in accordance with ASU No. 2015-03, "Interest – Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs." As of September 30, 2019, $230.0 million aggregate principal amount of the 2026 Convertible Notes and $460.0 million aggregate principal amount of the 2023 Convertible Notes was outstanding, for a total of $690.0 million aggregate principal amount outstanding.

12. Product Revenue, Net

The Company recognized net sales of Ocaliva of $61.5 million and $46.6 million for the three months ended September 30, 2019 and 2018, respectively, and $179.3 million and $124.9 million for the nine months ended September 30, 2019 and 2018, respectively.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
	(in thousands)		(in thousands)	
Product revenue, net:				
U.S.	$ 45,232	$ 36,684	$ 133,914	$ 99,697
ex-U.S.	16,313	9,897	45,372	25,211
Total product revenue, net	$ 61,545	$ 46,581	$ 179,286	$ 124,908

13. Stockholders' Equity

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 expire upon the earlier of (i) May 8, 2020 and (ii) the date that all of the 2019 Private Placement Shares have been sold or can be sold publicly under Rule 144 of the Securities Act on a single day. As of the date of this Quarterly Report on Form 10-Q, Samsara has not exercised any such registration rights.

The net proceeds to the Company from the 2019 Public Offering and the 2019 Concurrent Private Placement were approximately $227.2 million, after deducting underwriting discounts, commissions and estimated offering expenses of approximately $13.9 million.

2018 Public Offering and Concurrent Private Placement

On April 9, 2018, the Company issued and sold (i) 2,695,313 shares of common stock in a registered public offering (including 351,563 shares issued and sold upon the exercise in full of the underwriters' option to purchase additional shares), at a price to the public of $64.00 per share (the "2018 Public Offering") and (ii) 1,562,500 shares of common stock (the "2018 Private Placement Shares") in a concurrent private placement (the "2018 Concurrent Private Placement") exempt from the registration requirements of the Securities Act, at a purchase price per share equivalent to the price to the

public set in the 2018 Public Offering and pursuant to a securities purchase agreement (the "2018 Securities Purchase Agreement") that the Company entered into with the purchasers in the 2018 Concurrent Private Placement (the "Private Placement Purchasers"). Pursuant to the 2018 Securities Purchase Agreement, the Company granted to the Private Placement Purchasers certain registration rights which expired on April 4, 2019.

14. 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, 2019, the number of shares available for issuance under the 2012 Plan increased by 1,187,599 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, 2019 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, 2019 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, 2019 was determined utilizing the Monte Carlo simulation method.

The following table summarizes stock option activity during the nine months ended September 30, 2019:

	Number of Options (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2018	1,873	$ 97.63	7.5	$ 45,381
Granted	484	$ 105.55	—	$ —
Exercised	(69)	$ 36.59	—	$ —
Cancelled/forfeited	(148)	$ 92.40	—	$ —
Expired	(54)	$ 165.26	—	$ —
Outstanding at September 30, 2019	2,086	$ 97.93	7.4	$ 11,747
Expected to vest	985	$ 91.42	8.7	$ 2,533
Exercisable	1,101	$ 103.75	6.3	$ 9,214

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, 2019, the total compensation cost related to non-vested option awards not yet recognized is approximately $54.9 million with a weighted average remaining vesting period of 1.2 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,	
	2019	2018
Volatility	87.0 - 89.9 %	58.9 - 75.8 %
Expected term (in years)	5.5 - 6.0	6.0
Risk-free rate	1.4 - 2.9 %	1.8 - 2.9 %
Expected dividend yield	— %	— %

Effective January 1, 2019, the Company changed its expected volatility assumption to be estimated based on the historical stock price volatility of the Company over the expected term given the availability of sufficient historical trading

data. In prior years, the expected volatility was estimated based on historical volatility information of publicly-traded peer companies.

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, 2019:

	Number of Awards (in thousands)	Weighted Average Grant Date Fair Value
Non-vested awards at December 31, 2018	773	$ 76.10
Granted	354	$ 110.05
Vested	(261)	$ 78.71
Forfeited	(83)	$ 85.67
Non-vested awards at September 30, 2019	783	$ 89.57

As of September 30, 2019, there is approximately $53.4 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 1.4 years.

During the nine months ended September 30, 2019, the Company granted a total of 57,800 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 $2.0 million of stock-based compensation related to such PRSUs during the nine months ended September 30, 2019.

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.

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,	
	2019	2018	2019	2018
	(in thousands)		(in thousands)	
Selling, general and administrative	$ 10,104	$ 9,436	$ 33,029	$ 28,875
Research and development	3,026	2,558	9,780	9,540
Total stock-based compensation	$ 13,130	$ 11,994	$ 42,809	$ 38,415

15. Commitments and Contingencies

Legal Proceedings

The Company is involved in various disputes, governmental inquiries and investigations, legal proceedings and litigation in the course of its business, including the matters described below and, from time to time, intellectual property, 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.

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. Separately, 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. 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.

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.

In May 2018, the Company received a subpoena from the SEC requesting information in connection with the Company's patient assistance program and certain of the Company's commercial activities. The SEC's letter enclosing the subpoena states that the investigation and the subpoena do not mean that the Company or anyone else has broken the law, or that the SEC has a negative opinion of any person, entity or security. The Company is cooperating fully with the SEC in this matter. At this time, the Company is unable to predict whether any proceeding may be instituted in connection with the subpoena, or the outcome of any such proceeding, if instituted.

16. 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, 2019 and 2018, 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 basic and diluted 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 of September 30, 2019 and 2018, as the inclusion thereof would have been anti-dilutive:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
	(in thousands)		(in thousands)	
Convertible Notes	4,435	2,316	4,435	2,316
Options	2,086	1,997	2,086	1,997
Restricted stock units	597	447	597	447
Total	7,118	4,760	7,118	4,760

17. Subsequent Events

Sumitomo Agreement

On October 25, 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.

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, 2018 (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 a 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 and 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 to confirm the clinical benefit of OCA. OCA also achieved the primary endpoint in a Phase 2b clinical trial for the treatment of NASH 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 announced the submission of a New Drug Application ("NDA") to the FDA seeking approval of OCA for NASH. We have requested a priority review designation for the NDA which, if granted, would result in an anticipated six-month review period. We also currently intend to file a Marketing Authorization Application ("MAA") with the European Medicines Agency ("EMA") for OCA for NASH in the fourth quarter of 2019. In addition, we continue to work towards expanding our overall NASH development program with additional trials and studies, including our ongoing Phase 3 trial in NASH patients with compensated cirrhosis, known as the REVERSE trial. In August 2019, we announced the expansion of the REVERSE trial from 540 patients to approximately 900 patients and the extension of the trial from 12 to 18 months. The primary histologic endpoint of fibrosis improvement with no worsening of NASH was not modified.

As part of our product development activities, we expect to continue to invest in evaluating the potential of OCA in other progressive non-viral liver diseases beyond PBC and NASH. We also intend to study OCA in combination with bezafibrate, a pan-peroxisome proliferator-activated receptor ("PPAR") agonist, in patients with PBC and potentially other liver diseases. In addition, we have other compounds in early stages of research and development in our pipeline.

Recent Developments

OCA for NASH

In September 2019, we announced the submission of an NDA to the FDA seeking approval of OCA for NASH. We have requested a priority review designation for the NDA which, if granted, would result in an anticipated six-month review period.

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.

Effective January 1, 2018, we began recognizing revenue under Accounting Standards Codification ("ASC") Topic 606, *Revenue from Contracts with Customers* ("ASC 606"). The core principle of this revenue standard is that a company should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The following five steps are applied to achieve that core principle:

- Step 1: Identify the contract with the customer
- Step 2: Identify the performance obligations in the contract
- Step 3: Determine the transaction price
- Step 4: Allocate the transaction price to the performance obligations in the contract
- Step 5: Recognize revenue when the company satisfies a performance obligation

Product Revenue, Net

We provide the right of return to our customers for unopened product for a limited time before and after its expiration date. Prior to July 2017, given our limited sales history for Ocaliva and the inherent uncertainties in estimating product returns, we determined that the shipments of Ocaliva made to our customers did not meet the criteria for revenue recognition at the time of shipment. Accordingly, we recognized revenue when the product was sold through by our customers, provided all other revenue recognition criteria were met. We invoiced our customers upon shipment of Ocaliva to them and recorded accounts receivable, with a corresponding liability for deferred revenue equal to the gross invoice price. We then recognized revenue when Ocaliva was sold through as specialty pharmacies dispensed product directly to the patients (sell-through basis). We re-evaluated our revenue recognition policy in the third quarter of 2017, which included the accumulation and review of customer-related transactions since our commercial launch in the second quarter of 2016. We concluded we had accumulated sufficient data to reasonably estimate product returns and, therefore, began to recognize revenue at the time of shipment to our customers (sell-in basis).

Under 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 $61.5 million and $46.6 million for the three months ended September 30, 2019 and 2018, respectively, and $179.3 million and $124.9 million for the nine months ended September 30, 2019 and 2018, respectively.

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 May 2014, Sumitomo Dainippon exercised the Country Option in part to add Korea as part of its licensed territories and paid us a $1.0 million upfront fee in connection therewith. In February 2018, we and Sumitomo Dainippon entered into Amendment No. 3 (the "Sumitomo Amendment") to the Original Sumitomo Agreement (as amended, the "Sumitomo Agreement"), pursuant to which (i) Sumitomo Dainippon agreed to return the rights to develop and commercialize OCA in Japan and Korea and waived its rights to the Country Option, (ii) we agreed to forego any further milestone or royalty payments relating to the development and commercialization of OCA in Japan and Korea and (iii) certain milestone payment obligations with respect to the development and commercialization of OCA were adjusted. 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.

As of September 30, 2019, we had achieved $6.0 million of development milestones under the Sumitomo Agreement.

For accounting purposes, the upfront payments were recorded as deferred revenue and amortized over time and milestone payments are recognized once earned. The Company recognized licensing revenue of $0.4 million and $0.4 million for the three months ended September 30, 2019 and 2018, respectively, and $1.2 million and $1.6 million for the nine months ended September 30, 2019 and 2018, respectively, related to the amortization of the upfront payments under the Sumitomo Agreement. Included in licensing revenue for the nine months ended September 30, 2018 is $0.4 million related to the accelerated recognition, as a result of the Sumitomo Amendment, of the remaining portion of deferred revenue associated with the $1.0 million upfront payment that we received under the Original Sumitomo Agreement in connection with Sumitomo Dainippon's exercise of the Country Option with respect to Korea. We anticipate that we will recognize additional revenue of approximately $1.2 million in the fourth quarter of 2019, related to the amortization of 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, the preparation for the potential commercialization of OCA for 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, 2019 and 2018

The following table summarizes our results of operations for the three months ended September 30, 2019 and 2018:

	Three Months Ended September 30,	
	2019	2018
	(in thousands)	
Revenue:		
Product revenue, net	$ 61,545	$ 46,581
Licensing revenue	405	405
Total revenue	61,950	46,986
Operating expenses:		
Cost of sales	487	519
Selling, general and administrative	76,828	56,812
Research and development	60,168	47,941
Total operating expenses	137,483	105,272
Operating loss	(75,533)	(58,286)
Other income (expense):		
Interest expense	(11,795)	(7,671)
Other income, net	2,495	1,503
	(9,300)	(6,168)
Net loss	$ (84,833)	$ (64,454)

Revenues

Product revenue, net was $61.5 million and $46.6 million for the three months ended September 30, 2019 and 2018, respectively. For the three months ended September 30, 2019 and 2018, product revenue, net was comprised of U.S. Ocaliva net sales of $45.2 million and $36.7 million, respectively, and ex-U.S. Ocaliva net sales of $16.3 million and $9.9 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, 2019 and 2018, licensing revenue was $0.4 million and $0.4 million, respectively, in each case, related to the amortization of upfront payments under the Sumitomo Agreement.

Cost of sales

Cost of sales was $0.5 million and $0.5 million for the three months ended September 30, 2019 and 2018, respectively. Our cost of sales for the quarters ended September 30, 2019 and 2018 consisted primarily of packaging, labeling, materials and related expenses.

Selling, general and administrative expenses

Selling, general and administrative expenses were $76.8 million and $56.8 million for the three months ended September 30, 2019 and 2018, respectively. The $20.0 million net increase between periods was primarily driven by increases in expenses relating to our launch preparation activities associated with the potential approval and commercialization of OCA for NASH.

Research and development expenses

Research and development expenses were $60.2 million and $47.9 million for the three months ended September 30, 2019 and 2018, respectively. The $12.3 million net increase between periods was primarily driven by increases in OCA for NASH development program expenses and costs associated with the preparation of the NASH NDA submission.

Interest expense

Interest expense was $11.8 million and $7.7 million for the three months ended September 30, 2019 and 2018, respectively. For the three months ended September 30, 2019, interest expense related to 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. For the three months ended September 30, 2018, interest expense related only to the 2023 Convertible Notes.

Other income, net

Other income, net was $2.5 million and $1.5 million for the three months ended September 30, 2019 and 2018, 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, 2019 and 2018, 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, 2019 and 2018

The following table summarizes our results of operations for the nine months ended September 30, 2019 and 2018:

	Nine Months Ended September 30,	
	2019	2018
	(in thousands)	
Revenue:		
Product revenue, net	$ 179,286	$ 124,908
Licensing revenue	1,216	1,616
Total revenue	180,502	126,524
Operating expenses:		
Cost of sales	1,738	1,512
Selling, general and administrative	223,738	184,503
Research and development	178,163	144,028
Total operating expenses	403,639	330,043
Operating loss	(223,137)	(203,519)
Other income (expense):		
Interest expense	(29,518)	(22,769)
Other income, net	6,132	5,051
	(23,386)	(17,718)
Net loss	$ (246,523)	$ (221,237)

Revenues

Product revenue, net was $179.3 million and $124.9 million for the nine months ended September 30, 2019 and 2018, respectively. For the nine months ended September 30, 2019 and 2018, product revenue, net was comprised of U.S. Ocaliva net sales of $133.9 million and $99.7 million, respectively, and ex-U.S. Ocaliva net sales of $45.4 million and $25.2 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, 2019 and 2018, licensing revenue was $1.2 million and $1.6 million, respectively, in each case, related to the amortization of upfront payments under the Sumitomo Agreement. The decrease in licensing revenue related to the accelerated recognition in the first quarter of 2018 of certain upfront payments under the Original Sumitomo Agreement resulting from the Sumitomo Amendment.

Cost of sales

Cost of sales was $1.7 million and $1.5 million for the nine months ended September 30, 2019 and 2018, respectively. Our cost of sales for the nine months ended September 30, 2019 and 2018 consisted primarily of packaging, labeling, materials and related expenses.

Selling, general and administrative expenses

Selling, general and administrative expenses were $223.7 million and $184.5 million for the nine months ended September 30, 2019 and 2018, respectively. The $39.2 million net increase between periods was primarily driven by increases in expenses relating to our launch preparation activities associated with the potential approval and commercialization of OCA for NASH.

Research and development expenses

Research and development expenses were $178.2 million and $144.0 million for the nine months ended September 30, 2019 and 2018, respectively. The $34.2 million net increase between periods was primarily driven by increases in OCA for NASH development program expenses and costs associated with the preparation of the NASH NDA submission.

Interest expense

Interest expense was $29.5 million and $22.8 million for the nine months ended September 30, 2019 and 2018, respectively. For the nine months ended September 30, 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. For the nine months ended September 30, 2018, interest expense related only to the 2023 Convertible Notes.

Other income, net

Other income, net was $6.1 million and $5.1 million for the nine months ended September 30, 2019 and 2018, 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, 2019 and 2018, 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:

	Nine Months Ended September 30,	
	2019	2018
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ (176,129)	$ (187,902)
Investing activities	(254,876)	(84,999)
Financing activities	451,639	263,105
Effect of exchange rate changes	(505)	(835)
Net increase/(decrease) in cash and cash equivalents	$ 20,129	$ (10,631)

Operating Activities. 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.

Net cash used in operating activities of approximately $187.9 million during the nine months ended September 30, 2018 was primarily a result of our $221.2 million net loss and a net decrease in operating assets and liabilities of $21.7 million, partially offset by $38.4 million in stock-based compensation, $10.4 million for accretion of the discount on the 2023 Convertible Notes, $1.3 million for the loss on disposal of fixed assets and $3.6 million of depreciation.

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

For the nine months ended September 30, 2018, net cash used by investing activities primarily reflects the purchase of investment debt securities of $378.3 million, partially offset by the sales of investment debt securities of $293.3 million.

Financing Activities. 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.

Net cash provided by financing activities in the nine months ended September 30, 2018 consisted primarily of net proceeds of $261.4 million from our issuance and sale of common stock in a public offering and concurrent private placement in April 2018 and $1.7 million from the exercise of options to purchase common stock net of payments of employee withholding taxes related to stock-based awards.

Future Funding Requirements

As of September 30, 2019, we had $712.4 million in cash, cash equivalents and investment debt securities. We currently expect to continue to incur significant operating expenses in the fiscal year ending December 31, 2019. These expenses are planned to support, among other initiatives, the continued commercialization of Ocaliva for PBC in the United States and our other markets, launch preparation activities associated with the potential approval and commercialization of OCA for NASH, our continued clinical development of OCA for PBC and NASH and our other earlier stage research 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 and the initial phase of the anticipated U.S. launch of OCA for NASH, we may need to raise additional capital to fund our operating requirements beyond that period. Furthermore, in light of 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 forecast regarding the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results, including the costs to maintain our currently planned operations, could vary materially.

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;
- 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 timely and cost-effectively file for and obtain regulatory approval of our product candidates, including OCA for NASH, in the United States, Europe and our other target markets;
- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, such as the need for clinical outcomes data (and not just results based on achievement of

a surrogate endpoint), 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 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;
- 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 NASH, and our clinical trial activities;
- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to timely and successfully launch OCA for 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 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 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, 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 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 (the “SEC”), including our Annual Report.

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, our restated certificate of incorporation authorizes us to issue 45 million shares of common stock. Following the 2019 Public Offering, and after taking into account shares of common stock reserved for issuance upon the exercise of outstanding stock options, the vesting of outstanding restricted stock units (including performance restricted stock units) and the conversion of the Convertible Notes, together with shares of common stock available for future grants under our equity incentive plan, we have a limited number of remaining unreserved and authorized shares available for issuance, which could impact our ability to raise additional funds in the future. 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.

Off-Balance Sheet Arrangements

As of September 30, 2019, 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 15 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, 2018 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, 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. 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 Commercialization of Our Products" and "—Risks Related to the Development and the Regulatory Review and Approval of Our Products and Product Candidates" 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 $309.2 million, $360.4 million and $412.8 million for the years ended December 31, 2018, 2017 and 2016, respectively, and $246.5 million and $221.2 million for the nine months ended September 30, 2019 and 2018, 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 our licensing and collaboration agreements. At September 30, 2019, we had $712.4 million in cash, cash equivalents 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 the potential launch of OCA for 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, continue to commercialize Ocaliva for PBC, develop and seek and maintain regulatory approvals for OCA for NASH and other indications, and build-out the infrastructure in the United States and internationally necessary to support our product development and commercialization efforts. 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 anticipate that we will continue our Phase 4 COBALT trial of Ocaliva for PBC. We also expect to continue our Phase 3 clinical program of OCA for NASH, including our Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH through clinical outcomes in order to confirm clinical benefit and our Phase 3 REVERSE trial for NASH patients with compensated cirrhosis. We intend to evaluate the efficacy, safety and tolerability of bezafibrate in combination with OCA in patients with PBC in a Phase 2 study and to continue to develop OCA and our other existing product candidates, alone or in combination, for non-viral liver diseases in indications beyond NASH and PBC. Our overall development program for OCA for NASH is expected to include a number of trials, including clinical trials required to file for approval of OCA for NASH and to confirm clinical benefit. 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 delays in completing our clinical trials or the development of any of our product candidates.

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 advancing OCA through clinical development for multiple 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 and OCA for NASH, if approved. 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 approved products other than Ocaliva for PBC and OCA for 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, 2019, we had $712.4 million in cash, cash equivalents and investment debt securities. We currently expect to continue to incur significant operating expenses in the fiscal year ending December 31, 2019. These expenses are planned to support, among other initiatives, the continued commercialization of Ocaliva for PBC in the United States and our other markets, launch preparation activities associated with the potential approval and commercialization of OCA for NASH, our continued clinical development of OCA for PBC and NASH and our other earlier stage research 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 and the initial phase of the anticipated U.S. launch of OCA for NASH, we may need to raise additional capital to fund our operating requirements beyond that period. Furthermore, in light of 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 forecast regarding the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results, including the costs to maintain our currently planned operations, could vary materially.

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;
- 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 timely and cost-effectively file for and obtain regulatory approval of our product candidates, including OCA for NASH, in the United States, Europe and our other target markets;
- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), 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 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;

- 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 NASH, and our clinical trial activities;

- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to timely and successfully launch OCA for 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 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 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, 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 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, 2018.

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, our restated certificate of incorporation authorizes us to issue 45 million shares of common stock. Following the 2019 Public Offering, and after taking into account shares of common stock reserved for issuance upon the exercise of outstanding stock options, the vesting of outstanding restricted stock units (including performance restricted stock units) and the conversion of the Convertible Notes, together with shares of common stock available for future grants under our equity incentive plan, we have a limited number of remaining unreserved and authorized shares available for issuance, which could impact our ability to raise additional funds in the future. 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 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 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 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;
- 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 timely and cost-effectively file for and obtain regulatory approval of our product candidates, including OCA for NASH, in the United States, Europe and our other target markets;
- conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), 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 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;
- 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 NASH, and our clinical trial activities;
- our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to timely and successfully launch OCA for 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 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 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, 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 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, 2018.

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 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 an NDA, from the FDA, or an 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 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 alkaline phosphatase ("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 may be contingent upon the verification and description of clinical benefit in confirmatory trials. Our Phase 4 COBALT confirmatory outcomes trial may fail to show a clinical benefit for Ocaliva for PBC or may not satisfy applicable regulatory requirements for other reasons. As specified by the applicable post-marketing requirements, our COBALT trial includes subjects 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 have 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.

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 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 Phase 4 COBALT trial, may jeopardize the continued approval of Ocaliva for PBC in the United States, European Union and other jurisdictions.

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 an NDA or an 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 an NDA, the FDA may decide not to accept the submission for filing and review. Similarly, there may be delays in the EMA's review process following the submission of an MAA, or the EMA may determine that the submission does not support approval. In addition, in June 2016, eligible members of the electorate in the United Kingdom decided by referendum to leave the European Union, in what is often referred to as "Brexit". 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 NASH and our other product candidates.

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 authority 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, 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 regulatory approval for OCA for indications other than PBC, we will need to complete a number of additional clinical trials and studies. For example, in connection with our Phase 3 clinical program of OCA for NASH, we are currently conducting our Phase 3 REGENERATE trial in patients with liver fibrosis due to NASH through clinical outcomes in order to confirm clinical benefit and our Phase 3 REVERSE trial for NASH patients with compensated cirrhosis. 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 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 have submitted an NDA in the United States and currently intend to file for approval of OCA for NASH 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 NASH patients with liver fibrosis on an accelerated or conditional basis, or at all. Our Phase 3 REGENERATE trial remains blinded after the interim analysis and will continue to follow patients until the occurrence of a pre-specified number of adverse liver-related clinical events, including progression to cirrhosis, to confirm clinical benefit on a post-marketing basis, if approved.

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 an NDA. Furthermore, for full approval of an 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, including the NDA we submitted for NASH in September 2019. 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 support the approval of the product candidate. In such a case, the FDA may issue a complete response

letter that may require that we conduct and/or complete additional clinical trials and preclinical studies or provide other additional information or data before it will reconsider our application for approval. Any such requirements may be substantial, expensive and time-consuming, and there is no guarantee that the FDA will ultimately decide that any such regulatory application supports the approval of the product candidate. The FDA may also refer any regulatory application, such as the NDA we submitted in September 2019 or any other regulatory application we may file for OCA for NASH, 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 to confirm the clinical benefit of OCA for 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 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 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. If any such trial fails to show such adequate correlation, 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. 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 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 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 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. In November 2018, the EMA issued draft regulatory guidance 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 data supports the submission of an MAA with the EMA. However, the data that we submit to the EMA may not ultimately be found by the EMA to be sufficient for marketing approval. 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 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.

While OCA received breakthrough therapy designation from the FDA in January 2015 for the treatment of NASH patients with liver fibrosis and we have submitted an NDA in the United States and currently intend to file in Europe for approval of OCA for NASH based on the results from the 18-month interim 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 NASH patients with liver fibrosis on an accelerated or conditional basis, or at all. In addition, our regulatory submission may not be accepted by the FDA for review and, even if accepted for review, there may be delays in the FDA's review process or the FDA may determine that our submission does not support the approval of OCA for the treatment of NASH. Similarly, there may be delays in the EMA's review process or the EMA may determine that our submission does not support the approval of OCA for the treatment of NASH. Before granting approval, 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.

If we continue the development of OCA for primary sclerosing cholangitis ("PSC"), we may seek marketing approval based on a surrogate endpoint. While the EMA issued draft regulatory guidance in November 2018, the FDA has not issued formal guidance regarding a validated surrogate endpoint as a basis for seeking approval in PSC. Identifying an acceptable surrogate endpoint may take longer than we expect and any surrogate endpoint we select may ultimately not be accepted by the FDA, EMA or other applicable regulatory authorities.

Prior to any approval of OCA for 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, the relevant labeling may include restrictions, limitations and/or warnings that could impact the commercial success of OCA or our other product candidates in the applicable 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 Pediatric Investigation Plan 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. Consequently, we do not know whether our current or future clinical trials or studies of OCA or our other product candidates will begin or 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, REGENERATE is 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, 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.

Additionally, we have in the past occasionally experienced difficulties enrolling and retaining patients enrolled in our clinical trials. Difficulties in enrolling and retaining patients 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.

Clinical 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 efficacy for any indication, we will not be able to obtain 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 completion and results of post-marketing clinical trials, including our Phase 4 COBALT trial. We cannot assure you that these trials will demonstrate a correlation of the surrogate endpoint therapeutic response in patients taking Ocaliva for PBC with a significant reduction in adverse clinical outcomes over time.

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 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. While OCA received breakthrough therapy designation from the FDA in January 2015 for the treatment of NASH patients with liver fibrosis and we have submitted an NDA in the United States and currently intend to file in Europe for approval of OCA for 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 NASH patients with liver fibrosis 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 is ongoing to confirm the clinical benefit of OCA for 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. These events and any safety concerns associated with Ocaliva, perceived or real, may adversely affect the successful development and commercialization of our product candidates and lead to a loss of revenues.

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). 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 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.

In our Phase 2 AESOP trial of OCA for PSC, pruritus was the most common adverse event, occurring in 46% of patients on placebo, 60% of patients in the OCA 1.5 mg to 3 mg group and 67% of patients in the OCA 5 mg to 10 mg group, with the severity increasing with dose. One (4%) patient in the OCA 1.5 mg to 3 mg group and three (12%) patients in the OCA 5 mg to 10 mg group discontinued OCA due to pruritus compared to none in the placebo group.

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 will be 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 uptitration, 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 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 NASH patients with fibrosis or increase the likelihood that OCA will be granted marketing approval for NASH patients with fibrosis. 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 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 could 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.

We currently have an agreement with PharmaZell GmbH for the manufacture and commercial supply of Ocaliva and, if approved, OCA for NASH. While we have procured supplies for the commercialization of Ocaliva for PBC and, if approved, OCA for NASH, we may not be able to procure sufficient supplies of Ocaliva and, if approved, OCA for NASH on an ongoing basis. We have engaged in activities to qualify additional or back-up suppliers, but these suppliers may not be able to meet our long-term commercial supply requirements for Ocaliva or, if approved, OCA for NASH or other indications on acceptable terms, or at all. We do not have agreements for long-term supplies of any of our other product candidates. 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 must be the subject of a satisfactory inspection before the FDA or regulators in other jurisdictions approve the product candidate manufactured at that facility. 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.

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 NASH, are approved and our contract manufacturers fail to deliver the required commercial quantities of 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, 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 and other 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, 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 discovers 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.

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.

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. These events, the revised label, any future label changes that may be required by the FDA or other relevant regulatory authorities and any safety concerns associated with Ocaliva, perceived or real, may materially and adversely affect our Ocaliva commercialization efforts and, consequently, our financial condition and results of operations.

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 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 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 pharmaceuticals 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 and requirements for substitution of generic products. 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, we do not know if OCA for NASH will obtain and maintain broad acceptance from third-party payors, if approved. 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 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 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 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 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 NASH, if approved, or our other future approved products, if any.

Legislative 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 removes the financial penalties for people who do not carry health insurance commencing in 2019 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 whether the ACA will undergo additional revisions, and we cannot predict the impact of any future modifications. 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. We cannot predict the success of any such current or future federal or state legislative efforts.

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. 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 considered by the Trump administration and the United States Congress.

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 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 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.

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 label approved 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; and
- potential product liability claims.

In addition, the potential market opportunity for Ocaliva for PBC, OCA for 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 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 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 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 is our first product launch. We are commercializing Ocaliva for PBC using a combination of our internal commercial organization, a contract sales organization and third-party distributors depending on the jurisdiction. We are developing our commercialization strategy for OCA for 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 additional amounts of financial and management resources.

Recruiting and training a commercial organization is expensive, time-consuming and could delay any product launch. If the commercial launch of an approved 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 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.

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. For example, in May 2018, we received a subpoena from the SEC requesting information in connection with our patient assistance program and certain of our commercial activities.

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 or sales organizations, including our contract sales organization, to the extent that they do not comply with applicable laws and regulations. If we or our contract sales organization fail to comply with any of these laws and regulations, we could be subject to a range of penalties, including criminal and significant civil penalties, fines, damages, 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. For example, in early 2019, the U.S. Department of Health and Human Services issued a proposed rule that, if finalized, would modify the discount safe harbor under the federal anti-kickback statute to eliminate protection for certain drug discounts paid by manufacturers to plan sponsors under Medicare Part D or Medicaid managed care organizations or the pharmacy benefit managers working with these organizations.

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.

Finally, we must offer discounted pricing or rebates on Ocaliva 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 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 or effectively commercialize products, product candidates or technologies 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 or a failure to obtain the necessary regulatory approvals.

If we are unable to enter into new arrangements or maintain our existing 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 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 currently 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 able to successfully commercialize OCA for NASH.

The completion of development, securing of approval and commercialization of OCA for additional indications such as 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 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 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 active pharmaceutical ingredient and finished drug product for our commercial sales and for our clinical trials and preclinical studies and a contract sales organization for the commercialization of Ocaliva in certain jurisdictions. 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 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 the past, we experienced difficulties with a third-party contract manufacturer for OCA, including delays in receiving adequate clinical trial supplies as and when requested. We subsequently replaced this manufacturer, but it is possible that we could experience similar difficulties in the future. 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.

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 expect to compete with include 3-V Biosciences, Inc., 89bio, Inc., Allergan plc, Acorda Therapeutics, Inc., Affimune Limited, Akcea Therapeutics, Inc., Akero Therapeutics, Inc., Arrowhead Pharmaceuticals, Inc., AstraZeneca plc, Boehringer Ingelheim GmbH, Bristol-Myers Squibb Company, Can-Fite BioPharma Ltd., Celgene Corporation, Cirius 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., 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., Novartis AG, Novo Nordisk A/S, NuSirt Biopharma, Inc., Oramed Pharmaceuticals Inc., Pfizer Inc., Poxel SA, Second Genome, Inc., Sinew Pharma 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 SA has an ongoing Phase 3 clinical trial of elafibranor, a dual PPAR alpha/delta agonist, in NASH. Genfit is also 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 acquired from Nimbus Therapeutics, LLC, and cilofexor, an FXR agonist, in NASH patients. Gilead Sciences, Inc. is also studying a number of compounds in other liver diseases including PBC. Allergan plc has an ongoing Phase 3 clinical trial of cenicriviroc, a dual CCR2 and CCR5 inhibitor, for the treatment of NASH. CymaBay Therapeutics, Inc. is studying seladelpar, a peroxisome

proliferator-activated receptor δ agonist, for the treatment of PBC and NASH. A number of other companies have trials in PBC, NASH and other liver diseases that we are targeting.

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. Various other treatments, both approved and unapproved, have been used in the other indications we are targeting.

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 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 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 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, a contract sales organization and third-party distributors depending on the jurisdiction. Our 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 far-reaching anti-bribery and anti-corruption legislation in the United Kingdom, including the U.K. Bribery Act, and elsewhere and 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 trade restrictions;
- differing regulatory requirements for drug approvals internationally and the inability to obtain necessary foreign regulatory, pricing or reimbursement approvals for our products in a timely manner, or at all;
- uncertainty regarding the collectability of accounts receivable;
- difficulties in staffing and managing international operations;
- potentially reduced protection for our intellectual property rights;
- potential third-party patent rights in countries outside of the United States;
- the potential for so-called “parallel importing,” which is what occurs when a local seller opts to import goods from another jurisdiction with relatively low prices, rather than buying them locally;
- 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;
- compliance with tax, employment, immigration and labor laws applicable to our employees working or traveling abroad;
- 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, or natural disasters, including earthquakes, volcanoes, typhoons, floods, hurricanes and fires; 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.

In June 2016, eligible members of the electorate in the United Kingdom decided by referendum to leave the European Union, in what is often referred to as Brexit. 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 for years. Brexit could, among other outcomes, disrupt the free movement of goods, services and people between the United Kingdom and the European Union, result in changes 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 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 U.K. 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. 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, store and transmit large amounts of confidential information, including intellectual property, proprietary business information and personally identifiable information. It is

critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential 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. A security breach or privacy violation that leads to disclosure or modification of, or prevents access to, personally identifiable information or other protected information could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, 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 other regulatory penalties because of lost or misappropriated information. 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 prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information, trade secrets or other intellectual property. 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.

Our information security systems are subject to laws and regulations, or may become subject to new laws and regulations, requiring that we take measures to protect the privacy and security of certain information we gather and use in our business. For example, HIPAA, and its implementing regulations impose, among other requirements, certain regulatory and contractual requirements regarding the privacy and security of personal health information. In addition to HIPAA, numerous other federal and state laws, including, without limitation, 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.

Various foreign countries where we may process personal information also have, or are developing, laws governing the collection, use, disclosure and storage of personal information. 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.

In July 2016, U.S. and European Commission officials adopted a new framework called the EU-U.S. Privacy Shield to govern cross-border flows of personal information. We adopted the EU-U.S. Privacy Shield and have certified to its requirements since October 2016. In May 2018, the General Data Protection Regulation (the “GDPR”) took effect in the European Union. The GDPR imposes more stringent data protection requirements, and provides for greater penalties for noncompliance, than previous EU data protection legislation. Although the GDPR applies across the European Union without the need for local implementing legislation, local data protection authorities retain the ability to interpret the GDPR, which has the potential to create inconsistencies on a country-by-country basis. In addition, we do not know the extent of the impact that Brexit may have on our ability to transfer personal information between EU member states and the United Kingdom and we may need to develop new 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 actively employing the EU-U.S. Privacy Shield and the Swiss-U.S. Privacy Shield as a means to legitimize the transfer of personal information from the European Union and Switzerland to the United States, and are engaging in activities to comply with the GDPR requirements, we may be unsuccessful in these efforts. In addition, if currently available mechanisms utilized for the transfer of personal information, such as the EU-U.S. Privacy Shield and the Swiss-U.S. Privacy Shield, are invalidated in litigation or otherwise, we may not be able to employ suitable mechanisms to continue such transfers and our ability to conduct our business may be materially 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. 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 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.

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 NASH. We may experience difficulties in managing our significant growth.

We have significantly expanded our operations, including the size of our employee base, and expect to continue to grow 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 product development programs, we may need to increase our product development, scientific and administrative headcount. We will also need to grow our commercial capabilities. 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.

In addition, in order to continue to meet our obligations as a public company and to support our anticipated longer-term growth, we will 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 and expand 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 scientific and clinical advisors and consultants who assist us in formulating our research, development, clinical and regulatory strategies. 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 in developing products or technologies that may compete with ours.

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, our business and results of operations would likely be materially and adversely affected.

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 inquires, 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.

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, 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 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.

If we engage in an in-license 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 or entering into a business combination 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.

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.

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, 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.

In late 2017, the United States enacted the Tax Cuts and Jobs Act of 2017 (the "TCJA"), which significantly changed U.S. tax law, including by implementing a reduction in the corporate tax rate to 21%, moving from a worldwide tax system

to a territorial system and imposing new or additional limitations on the deductibility of interest expense and executive compensation.

The impact on our effective income tax rate resulting from the above-mentioned factors and others may be significant and could adversely affect our results of operations.

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 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 our products such as Ocaliva and 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.

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 patentability 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.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make compounds that are similar to our products or product candidates but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our patents or pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies, or may duplicate any of our technologies;
- any patents that we obtain may not provide us with any competitive advantages or exclusivity in a particular product area or indication or for the length of time we have anticipated;
- we may not develop additional proprietary technologies that are patentable; 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 with claims directed to pharmaceutical compounds, pharmaceutical compositions, methods of making these compounds and methods of using these compounds in various indications. In addition, we are the owner of record of numerous pending U.S. and non-U.S. patent applications, and regularly pursue additional patent applications in various jurisdictions.

The issued composition of matter patents for OCA are expected to expire in 2022 at the earliest and 2036 at the latest if the appropriate maintenance, renewal, annuity, or other government fees are paid. Without patent protection, including patent protection covering the composition of matter of our products and product candidates, our ability to stop others from using or selling our products and product candidates may be limited.

Due to the patent laws of a country, the decisions of a patent examiner in a country or our own filing strategies, we may not obtain patent coverage for all of our products and product candidates or methods involving these candidates in the parent patent application. While we plan to pursue divisional patent applications or continuation patent applications in the United States and other countries to obtain claim coverage for inventions which were disclosed but not claimed in the parent patent application, we cannot be certain that such patents will be granted or that the scope of any patent granted will prevent third parties from selling the same or similar products.

If we do not obtain protection under the Hatch-Waxman Act and similar legislation outside of the United States by extending the patent terms and obtaining data 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 of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. 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 restoration or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened, our competitors may obtain approval of competing products following our patent expiration and our revenue could be reduced, possibly materially.

Our primary composition of matter patent for OCA expires in 2022. In light of the U.S. marketing approval of Ocaliva for PBC in May 2016, we applied for an extension of the patent term for this patent in the United States through 2027. In addition, 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 this patent in the European Union through 2027. We have received grants of SPC in Austria, Cyprus, Denmark, 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 composition of matter patents for OCA are expected to expire in 2022 at the earliest and 2036 at the latest if the appropriate maintenance, renewal, annuity, or other government fees are paid.

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 go to court or engage in other adversarial proceedings to stop another party from using the inventions claimed in any of our current or future patents, that individual or company 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 and would 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 using the inventions. In addition, the U.S. Supreme Court has modified some tests utilized by the USPTO in granting patents over the past 20 years, which may decrease the likelihood that we will be able to obtain patents 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 our products, or the manufacture or use of our product candidates, 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. The pharmaceutical and biotechnology industries have produced a proliferation of patents, 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 our products, product candidates or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. 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, defend an infringement action successfully or have infringed patents declared invalid, 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 agencies 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 of their former employers. If we are not able to adequately prevent disclosure of 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 confidential 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 pay 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 sheet, 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 our largest stockholder 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. In addition, Genextra has informed us that it has pledged shares of our common stock that it holds as collateral in connection with a margin loan. Enforcement against such collateral could materially and adversely affect the 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.

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. For example, in May 2018, we received a subpoena from the SEC requesting information in connection with our patient assistance program and certain of our commercial activities. 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 Annual Report on Form 10-K for the year ended December 31, 2018, the factors that may result in wide fluctuations in the price of our common stock include any:

- receipt of additional marketing authorizations for Ocaliva in our target markets or for our product candidates, including OCA for NASH;
- failure to successfully commercialize Ocaliva for PBC or our other approved products in the United States, Europe and our other target markets in which we have or may receive marketing authorization or our inability to maintain regulatory approval for Ocaliva or our other approved products in such markets;
- 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;
- inability to obtain additional funding;
- delay in filing an investigational new drug application, NDA, MAA or comparable submission for any of our product candidates, including OCA for NASH, and any adverse development or perceived adverse development with respect to the regulatory review of any such submission;
- failure to successfully develop, obtain regulatory approval of and, if approved, commercialize OCA for indications other than PBC, such as NASH, or any of our other product candidates;
- potential side effects associated with Ocaliva for PBC, OCA for NASH or our other product candidates;
- inability to obtain adequate product supply of Ocaliva, OCA 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 actions with respect to our products or product candidates 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 and market 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. These broad market and industry fluctuations, as well as general economic, political and market conditions such as 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 all 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. Furthermore, Genextra has informed us that it has pledged shares of our common stock that it holds as collateral in connection with a margin loan. Enforcement against such collateral could materially and adversely affect the price of our common stock and the Convertible Notes.

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.

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

We may raise additional 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.

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.

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 income tax purposes. The enactment of the TCJA in late 2017 modified the ability of companies to utilize 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. 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 similar rules. 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 carryforwards for U.S. federal, state, and foreign tax purposes.

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, 2019.

Period	Total Number of Shares Purchased (1)	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	Maximum Number (or Approximate Dollar Value) of Shares that May Yet Be Purchased Under the Plans or Programs
July 1, 2019 through July 31, 2019	2,801	$ 78.80	—	—
August 1, 2019 through August 31, 2019	1,493	$ 63.61	—	—
September 1, 2019 through September 30, 2019	—	—	—	—
Total	4,294	$ 73.52	—	—

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

Item 5. Other Information

On November 1, 2019, the Company entered into a third amendment (the "New York Lease Amendment") to the lease agreement (the "New York Lease") with Legacy Yards Tenant LP relating to the space leased for the Company's global corporate headquarters at 10 Hudson Yards in New York. The New York Lease Amendment (i) extends the term of the New York Lease through March 2022 and (ii) provides for the lease of an additional approximately 4,500 square feet beginning in January 2020 for a total of approximately 45,600 square feet of office space in New York.

The foregoing is only a summary description of the terms of the New York Lease Amendment, does not purport to be complete and is qualified in its entirety by reference to the New York Lease Amendment, a copy of which is filed as Exhibit 10.1 to this Quarterly Report on Form 10-Q and incorporated herein by reference.

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

Exhibit Number	Description of Exhibit
10.1	Third Amendment to Lease, dated November 1, 2019, between the Registrant and Legacy Yards Tenant LP
31.1	Certification of Principal Executive Officer required by Rule 13a-14(a) or Rule 15d-14(a).
31.2	Certification of Principal Financial Officer required by Rule 13a-14(a) or Rule 15d-14(a).
32.1(1)	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).
101	The following materials from the Registrant's Quarterly Report on Form 10-Q for the period ended September 30, 2019, formatted in Inline XBRL (Inline eXtensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets at September 30, 2019 (unaudited) and December 31, 2018

	(audited), (ii) Condensed Consolidated Statements of Operations for the three and nine-month periods ended September 30, 2019 and 2018 (unaudited), (iii) Condensed Consolidated Statements of Comprehensive Loss for the three and nine-month periods ended September 30, 2019 and 2018 (unaudited), (iv) Condensed Consolidated Statements of Changes in Stockholders' Equity for the three and nine-month periods ended September 30, 2019 and 2018 (unaudited), (v) Condensed Consolidated Statements of Cash Flows for the nine-month periods ended September 30, 2019 and 2018 (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).

(1) 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 the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, (whether made before or after the date of the Quarterly Report on Form 10-Q), irrespective of any general incorporation language contained 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 5, 2019

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

Date: November 5, 2019

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

THIRD AMENDMENT TO LEASE

This **THIRD AMENDMENT TO LEASE** (this "Amendment"), dated as of November 1, 2019 (the "Third Amendment Effective Date"), between **LEGACY YARDS TENANT LP**, a Delaware limited partnership ("Landlord"), having an address at c/o Related Companies, 60 Columbus Circle, 19th Floor, New York, New York 10023, and **INTERCEPT PHARMACEUTICALS, INC.**, a Delaware corporation ("Tenant"), having an address at 10 Hudson Yards, 37th Floor, New York, New York 10001.

WITNESSETH:

WHEREAS, pursuant to a Lease, dated as of December 7, 2016 (the "Original Lease"), by and between Landlord and Tenant, as amended by (i) that certain First Amendment to Lease, dated as of June 27, 2017 (the "First Amendment"), (ii) that certain letter agreement, dated December 31, 2017 (the "12/31/17 Letter Agreement"), (iii) that certain Second Amendment to Lease, dated as of June 22, 2019 (the "Second Amendment"), and (iv) that certain letter agreement, dated October 12, 2018 (the "10/12/18 Letter Agreement"; the Original Lease, as so amended, the "Lease"), Tenant is leasing from Landlord certain space in the building known as 10 Hudson Yards, located at the corner of 10th Avenue and 30th Street, New York, New York (the "Building"), as is more particularly described in the Lease; and

WHEREAS, Landlord and Tenant desire to further amend the Lease on the terms and conditions hereinafter set forth.

NOW, THEREFORE, Landlord and Tenant agree as follows:

1. Defined Terms. All capitalized terms used but not otherwise defined herein shall have the meanings ascribed to them in the Lease.

2. Extension of Term of 37th Floor Premises.

(a) The term of the Lease with respect to the 37th Floor Premises is hereby extended upon and subject to the terms, covenants and conditions of the Lease, as amended by this Amendment, for an extension term (the "37th Floor Extension Term") commencing on July 1, 2021 (the "37th Floor Extension Term Commencement Date") and ending on March 31, 2022 (the "37th Floor Expiration Date"), which date shall be deemed the Expiration Date for the 37th Floor Premises for all purposes under the Lease, unless sooner terminated in accordance with the terms of the Lease or pursuant to law.

(b) Tenant's leasing of the 37th Floor Premises during the 37th Floor Extension Term shall be on all of the terms and conditions of the Lease (as amended hereby), except that, from and after the 37th Floor Extension Term Commencement Date, Tenant shall pay Fixed Rent in respect of the 37th Floor Premises, at the times and in the manner set forth in the Lease, for the period commencing on the 37th Floor Extension Term Commencement Date and ending on the 37th Floor Expiration Date in an amount equal to $4,268,089.00 per annum (i.e., at the rate of $127.00 per rentable square foot of the 37th Floor Premises), payable in equal monthly installments of $355,674.08 (as appropriately prorated for any partial month during the 37th Floor Extension Term).

3. Second Extension of Term of 40th Floor Premises.

(a) The term of the Lease with respect to the 40th Floor Premises, as previously extended pursuant to the Second Amendment, is hereby further extended upon and subject to the terms, covenants and conditions of the lease, as amended by this Amendment, for a term (the "40th Floor Second Extension Term") commencing on January 1, 2020 (the "40th Floor Second Extension Term Commencement Date") and ending on March 31, 2022 (the "40th Floor Expiration Date"), which date shall be deemed the Expiration Date for the 40th Floor Premises for all purposes under the Lease, unless sooner terminated in accordance with the terms of the Lease or pursuant to law.

(b) Tenant's leasing of the 40th Floor Premises during the 40th Floor Second Extension Term shall be on all of the terms and conditions of the Lease (as amended hereby), except that, from and after the 40th Floor Second Extension Rent Commencement Date, Tenant shall pay Fixed Rent in respect of the 40th Floor Premises at the times and in the manner set forth in the Lease, for the period commencing on the 40th Floor Second Extension Rent Commencement Date and ending on the 40th Floor Expiration Date in an amount equal to $947,420.00 per annum (i.e., at the rate of $127.00 per rentable square foot of the 40th Floor Premises), payable in equal monthly installments of $78,951.67 (as appropriately prorated for any partial month during the 40th Floor Extension Term). For purposes of this Paragraph 3(b), the term "40th Floor Second Extension Rent Commencement Date" shall mean February 12, 2020.

4. Additional Term for 41st Floor Premises.

(a) Tenant previously leased from Landlord a portion of the 41st Floor of the building (the "41st Floor Premises"), substantially as shown shaded in blue on the floor plan attached hereto as Exhibit B. Tenant's lease for the 41st Floor Premises expired on October 31, 2018. Tenant desires to again lease from Landlord the 41st Floor Premises, and Landlord agrees to lease to Tenant the 41st Floor Premises, for the period (the "41st Floor Additional Term") commencing January 15, 2020 (the "41st Floor Additional Term Commencement Date") and ending March 31, 2022 (the "41st Floor Additional Term Expiration Date"), which date shall be deemed the Expiration Date for the 41st Floor Premises for all purposes under the Lease, unless sooner terminated in accordance with the terms of the Lease or pursuant to law.

(b) Tenant's leasing of the 41st Floor Premises during the 41st Floor Additional Term shall be on all of the terms and conditions of the Lease (as amended hereby), except that, from and after the 41st Floor Additional Rent Commencement Date, Tenant shall pay Fixed Rent in respect of the 41st Floor Premises at the times and in the manner set forth in the Lease, for the period commencing on the 41st Floor Additional Rent Commencement Date and ending on the 41st Floor Additional Term Expiration Date in an amount equal to $567,182.00 per annum (i.e., at the rate of $127.00 per rentable square foot of the 41st Floor Premises), payable in equal monthly installments of $47,265.17 (as appropriately prorated for any partial month during the 41st Floor Additional Term). For purposes of this Paragraph 4(b), the term "41st Floor Additional Rent Commencement Date" shall mean February 26, 2020.

(c) Tenant acknowledges and agrees that Tenant has inspected the 41st Floor Premises and that it shall accept vacant and "broom-clean" possession of the 41st Floor Premises in the condition which exists on the Third Amendment Effective Date (subject to reasonable wear and tear between the Third Amendment Effective Date and the 41st Floor Premises Commencement Date), and that Landlord has no obligation to perform any work or make any installations in order to prepare the 41st Floor Premises for Tenant's occupancy.

5. Additional Work Allowance for 40th Floor Premises and 41st Floor Premises.

(a) Landlord shall reimburse Tenant for costs incurred by Tenant for Tenant's Work performed at the 40th Floor Premises and/or the 41st Floor Premises ("Tenant's Additional Work") within 10 months after the Third Amendment Effective Date (the "Additional Work Reimbursement Period") up to (and in no event in excess of) an amount (the "Additional Work Allowance") equal to $238,520.00 (i.e., $20.00 per rentable square foot of the 40th Floor Premises and the 41st Floor Premises), upon the following terms and conditions:

(i) The Additional Work Allowance shall be payable to Tenant in installments as Tenant's Additional Work progresses, but in no event more frequently than monthly. Installments of the Additional Work Allowance shall be payable by Landlord within 30 days following Tenant's satisfaction of (or substantial compliance to Landlord's reasonable satisfaction with) each of the conditions required for disbursement set forth in this Paragraph 5(a), it being understood that minor or insubstantial deviations from any documentary requirements included in said conditions that are otherwise reasonably satisfactory to Landlord shall not result in a withholding of the installment of the Additional Work Allowance requested by Tenant.

(ii) Prior to the payment of any installment, Tenant shall deliver to Landlord a request for disbursement (each being hereinafter called a "Tenant Requisition"), which shall be accompanied by (1) invoices for Tenant's Additional Work performed or incurred since the last Tenant Requisition and disbursement of the Additional Work Allowance, (2) a certificate signed by Tenant's architect and an officer of Tenant certifying that to such architect's and officer's knowledge, Tenant's Additional Work and services represented by the aforesaid invoices have been satisfactorily completed in substantial accordance with the plans and specifications therefor approved by Landlord to the date of such certification, and have not been the subject of a prior disbursement of the Additional Work Allowance, and (3) lien waivers by architects, contractors, subcontractors and all materialmen for all such work and services (it being understood and agreed that conditional lien waivers shall be delivered for work which is the subject of Tenant Requisition in question and unconditional lien waivers shall be delivered for all completed work which was the subject of the previous Tenant Requisition). If any matter concerning a Tenant Requisition is disputed by Landlord, any undisputed portion thereof shall be funded by Landlord without limiting Landlord's rights to dispute the disputed portion, and such dispute with respect to such disputed portion shall be resolved by arbitration in accordance with the provisions of Section 8.09 of the Lease. Each installment payment of the Additional Work Allowance shall be limited to an amount equal to the amount requested by Tenant pursuant to clause (1) of this paragraph. In addition, if the amount requested by Tenant does not already reflect the Minimum Retainage against the amount requested by the applicable contractor or subcontractor, then Landlord shall be permitted to retain from each disbursement an amount equal to the Minimum Retainage of the amount requested to be disbursed by Tenant. "Minimum Retainage" means (1) 10% until at least 50% of Tenant's Additional Work is substantially complete and paid for and (2) 5% thereafter.

(iii) Tenant is not then in default either (x) of any monetary obligation under this Lease or (y) of any non-monetary obligation under this Lease (after, solely in the case of non-monetary obligations, the giving of required notices, if any, and the expiration of applicable cure periods, if any).

(iv) In no event shall more than 15% of the Additional Work Allowance be made available to Tenant for Tenant's soft costs of construction (including, without limitation, filing and permit fees and expenses, architecture, engineering and other consulting fees and expenses and moving expenses).

(b) "Tenant's Additional Work" means the alterations, installations and improvements to be performed by Tenant in the Premises to prepare the same for initial occupancy thereof. The Additional Work Allowance shall not be utilized by Tenant to purchase any furniture for the Premises or for any audio/video work performed by Tenant in connection with Tenant's Additional Work.

(c) The right to receive reimbursement for the cost of Tenant's Additional Work as set forth in this Paragraph 5 shall be for the exclusive benefit of Tenant, it being the express intent of the parties hereto that in no event shall such right be conferred upon or for the benefit of any third party, including, without limitation, any contractor, subcontractor, materialman, laborer, architect, engineer, attorney or any other person, firm or entity.

(d) Tenant shall not be entitled to deliver a Tenant Requisition for a disbursement of any portion of the Additional Work Allowance later than the date that is 60 days after the last day of the Additional Work Reimbursement Period (the "Additional Work Outside Requisition Date") and if Tenant shall fail to deliver a Tenant Requisition for a disbursement in connection with any Tenant's Additional Work by the Additional Work Outside Requisition Date, then Tenant shall waive Tenant's right to receive any payment in connection therewith.

(e) If Tenant satisfies all of the conditions to payment of the Additional Work Allowance in accordance with this Paragraph 5 and Landlord fails to pay to Tenant any amount of the Additional Work Allowance (subject to Landlord's right to directly pay certain costs to be reimbursed by Tenant to Landlord from the Additional Work Allowance as more particularly described in Paragraph 5(b) above) on or before the date on which the same is due and payable to Tenant under this Paragraph 5, and provided that such failure continues for 30 days after Tenant notifies Landlord of such failure (which notice shall contain a legend in not less than 14 point font bold upper case letters as follows: "THIS IS A NOTICE OF A CLAIMED OFFSET RIGHT GIVEN IN ACCORDANCE WITH PARAGRAPH 5(e) OF THE THIRD AMENDMENT TO LEASE"), then, subject to the further provisions of this Paragraph 5(e), Tenant may set off such amount against the next installments of Rent coming due under this Lease. Landlord shall have the right within such 30-day period to deliver written notice to Tenant that Landlord disputes, in good faith, Tenant's entitlement to the amount claimed by Tenant, together with a reasonably detailed explanation of the reasons therefor, it being agreed that if Landlord timely delivers such written notice, then Tenant shall not have the right to set off such amounts until the dispute is resolved in accordance with the further provisions of this Paragraph 5(e). If Landlord fails to deliver such written notice to Tenant within such 30-day period, Landlord shall be deemed to have accepted Tenant's entitlement to the amount claimed by Tenant. In the event Landlord does deliver such written notice to Tenant within such 30-day period as provided above, the parties shall, in good faith, resolve such dispute(s) in a timely manner. Either party may submit any such dispute that remains unresolved for more than 30 days to arbitration in accordance with the provisions of Section 8.09 of the Lease. Any other dispute with respect to the payment of the Additional Work Allowance shall also be resolved by arbitration in accordance with the provisions of Section 8.09 of the Lease. If any such dispute is resolved in favor of Tenant, then the amount in dispute shall be paid to Tenant within 10 days after the determination of the arbitrator, failing which Tenant may give to Landlord 5 Business Days' notice of Tenant's intent to offset the amount due to Tenant against the next installments of Rent due under this Lease (which notice shall contain a legend in

not less than 14 point font bold upper case letters as follows: "THIS IS A NOTICE OF A CLAIMED OFFSET RIGHT GIVEN IN ACCORDANCE WITH PARAGRAPH 5(e) OF THE THIRD AMENDMENT TO LEASE") and if Landlord does not, within such 5 Business Day period, pay such amount to Tenant, then Tenant may set off such amount against the next installments of Rent coming due under this Lease.

6. Amendments. From and after the Third Amendment Effective Date, the following provisions of the Lease are amended as hereinafter set forth:

(a) Exhibit B to the Lease (as previously amended) shall be deleted in its entirety and replaced with Exhibit B attached hereto.

(b) The definitions of "37th Floor Expiration Date" and "40th-41st Floor Expiration Date" in Section 1.02 of the Original Lease (as previously amended) are hereby deleted in their entirety and replaced with the following:

"(x) with respect to the 37th Floor Premises, on March 31, 2022 (the "37th Floor Expiration Date"), (y) with respect to the 40th Floor Premises, on March 31, 2022 (the "40th Floor Expiration Date") and (z) with respect to the 41st Floor Premises, on March 31, 2022 (the "41st Floor Expiration Date"; the 37th Floor Expiration Date, the 40th Floor Expiration Date and the 41st Floor Expiration Date, collectively or individually, as the context requires, is call the "Expiration Date")."

(c) The reference to "2.89%" in the last sentence of Section 2.04(e) (as previously amended) is hereby deleted and replaced with "2.68%".

(d) The references to "10 tons" in the parenthetical clause of the first sentence of Section 3.01(f) and in the parenthetical clause of the fifth sentence of Section 3.01(f) (as previously amended) are hereby deleted and replaced with "7.5 tons".

(e) 37th Floor Premises Renewal Right. The date "December 1, 2019" in Paragraph 4(b)(i) of the Second Amendment is changed to "September 1, 2020".

7. Brokerage. Each party represents to the other that such party has dealt with no broker (except that Landlord represents to Tenant that it has dealt with ERY Manager) in connection with this Amendment. Each party shall indemnify and hold the other harmless from and against all loss, cost, liability and expense (including, without limitation, reasonable attorneys' fees and disbursements) arising out of any claim for a commission or other compensation by any broker (other than ERY Manager) who alleges that it has dealt with the indemnifying party in connection with this Amendment or the Project; provided, however, that Tenant does not indemnify Landlord with respect to any claim for a commission or other compensation by any broker arising out of a brokerage agreement or commission agreement heretofore entered into between Landlord and a broker with respect to the Lease that is amended by this Amendment. Landlord shall pay any commission that may be due to ERY Manager pursuant to the terms and conditions of a separate agreement between Landlord and ERY Manager.

8. No Other Changes. Except as expressly set forth in this Amendment, the Lease shall remain unmodified and in full force and effect, and the Lease as modified herein is ratified and confirmed. All references in the Lease to "this Lease" shall hereafter be deemed to refer to the Lease as amended by this Amendment.

9. Miscellaneous. This Amendment contains the entire agreement of the parties with respect to the subject matter hereof and all prior negotiations, understandings or agreements between the parties with respect to the subject matter hereof are merged herein. This Amendment may be executed in counterparts each of which shall be an original, and all of which counterparts taken together shall constitute one and the same agreement. Landlord and Tenant each hereby represents to the other that, to the best of its knowledge, there exist no defaults under the Lease nor do there exist any events which, with the giving of notice or the passage of time, would constitute defaults by the other party under the Lease. The Lease and all of the terms, covenants and provisions thereof, as amended hereby, are and shall remain in full force and effect and are hereby ratified and confirmed in all respects. Neither this Amendment nor the Lease (as amended hereby) may be further modified, amended, changed or terminated orally, but only by an agreement in writing signed by the party against whom enforcement of the modification, amendment, change or termination is sought. This Amendment shall be binding upon and inure to the benefit of Landlord and Tenant and their respective permitted successors and assigns. In case of any conflict between the terms and provisions of this Amendment and the terms and provisions of the Lease, the terms and provisions of this Amendment shall control.

[Remainder of page intentionally left blank; signatures on following page]

IN WITNESS WHEREOF, Landlord and Tenant have duly executed this Amendment as of the day and year first above written.

LANDLORD:

LEGACY YARDS TENANT LP

By: Legacy Yards Tenant GP LLC, its general partner

By: /s/ Andrew Rosen
Name: Andrew Rosen
Title: VP

TENANT:

INTERCEPT PHARMACEUTICALS, INC.

By: /s/ Mark Pruzanski
Name: Mark Pruzanski
Title: President & CEO

EXHIBIT B

FLOOR PLANS OF PREMISES

[attached]

Exhibit 31.1

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 5, 2019

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

Exhibit 31.2

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 5, 2019

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

Exhibit 32.1

CERTIFICATION

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), 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, 2019, 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 5, 2019

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

Date: November 5, 2019

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.