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 June 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 001-33451

Albireo Pharma, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**90-0136863**
(State or other jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**
10 Post Office Square, Suite 502 South, Boston, MA	**02109**
(Address of principal executive offices)	**(Zip code)**

Registrant's telephone number, including area code: (857) 254-5555

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	ALBO	The Nasdaq Capital 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

As of August 1, 2019, there were 12,685,326 shares of Common Stock, $0.01 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This quarterly report includes forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, which we refer to as the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, which we refer to as the Exchange Act, that relate to future events or to our future operations or financial performance. Any forward-looking statement involves known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from any future results, levels of activity, performance or achievements expressed or implied by such forward-looking statement. Forward-looking statements include statements, other than statements of historical fact, about, among other things:

- the progress, number, scope, cost, duration or results of our development activities, nonclinical studies and clinical trials of odevixibat (formerly known as A4250), elobixibat, A3384 or any of our other product candidates or programs, such as the target indication(s) for development or approval, the size, design, population, conduct, cost, objective or endpoints of any clinical trial, or the timing for initiation or completion of or availability of results from any clinical trial (including PEDFIC 1, our Phase 3 clinical trial of odevixibat in patients with progressive familial intrahepatic cholestasis, or PFIC), for submission or approval of any regulatory filing, or for meeting with regulatory authorities;

- the potential benefits that may be derived from any of our product candidates;

- the timing of and our ability to obtain and maintain regulatory approval of our existing product candidates, any product candidates that we may develop, and any related restrictions, limitations, or warnings in the label of any approved product candidates;

- any payment that HealthCare Royalty Partners III, L.P., or HCR, or EA Pharma Co., Ltd., or EA Pharma, may make to us or any other action or decision that EA Pharma may make concerning elobixibat or our business relationship;

- our future operations, financial position, revenues, costs, expenses, uses of cash, capital requirements, our need for additional financing or the period for which our existing cash resources will be sufficient to meet our operating requirements; or

- our strategies, prospects, plans, expectations, forecasts or objectives.

Words such as, but not limited to, “believe,” “expect,” “anticipate,” “estimate,” “forecast,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “targets,” “likely,” “will,” “would,” “could,” “should,” “continue,” “scheduled” and similar expressions or phrases, or the negative of those expressions or phrases, are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Although we believe that we have a reasonable basis for each forward-looking statement contained in this report, we caution you that these statements are based on our estimates or projections of the future that are subject to known and unknown risks and uncertainties and other important factors that may cause our actual results, level of activity, performance, experience or achievements to differ materially from those expressed or implied by any forward-looking statement. Actual results, level of activity, performance, experience or achievements may differ materially from those expressed or implied by any forward-looking statement as a result of various important factors, including our critical accounting policies and risks and uncertainties relating, among other things, to:

- the design, size, duration and endpoints for, and results from, PEDFIC 1, our Phase 3 clinical trial of odevixibat in patients with PFIC or our related extension study, or any other trials that will be required to obtain marketing approval for odevixibat to treat patients with PFIC or any other pediatric cholestatic liver disease, for elobixibat to treat nonalcoholic steatohepatitis, or NASH, or for A3384 to treat bile acid malabsorption, or BAM;

- whether favorable findings from clinical trials of odevixibat to date, including findings in indications other than PFIC, will be predictive of results from future clinical trials, including the trials comprising our Phase 3 PFIC program for odevixibat;

- whether either or both of the U.S. Food and Drug Administration, or FDA, and European Medicines Agency, or EMA, will determine that the primary endpoint and treatment duration of the double blind Phase 3 trial in patients with PFIC are sufficient, even if such primary endpoint is met with statistical significance, to support approval of odevixibat in the United States or the European Union, to treat PFIC, a symptom of PFIC, a specific PFIC subtype(s) or otherwise;

- the outcome and interpretation by regulatory authorities of an ongoing third-party study pooling and analyzing long-term PFIC patient data;

- the timing for initiation or completion of, or for availability of data from, the trials comprising the Phase 3 PFIC program for odevixibat, and the outcomes of such trials;

- delays or other challenges in the recruitment of patients for the double blind Phase 3 trial of odevixibat;

- whether odevixibat will meet the criteria to receive a rare pediatric disease priority review voucher from the FDA when applicable, whether a rare pediatric disease priority review voucher that we may receive in the future for odevixibat, if any, will be valuable to us, and, if necessary, whether the rare pediatric disease priority review voucher program will be renewed beyond 2020;

- the competitive environment and commercial opportunity for a potential treatment for PFIC and other orphan pediatric cholestatic liver diseases;

- the conduct and results of clinical trials and nonclinical studies and assessments of odevixibat, elobixibat, A3384 or any of our other product candidates and programs, including the performance of third parties engaged to execute them and difficulties or delays in patient enrollment and data analysis;

- the medical benefit that may be derived from odevixibat, elobixibat, A3384 or any of our other product candidates;

- the extent to which our agreements with HCR and EA Pharma for elobixibat generate nondilutive income for us;

- the timing and success of submission, acceptance and approval of regulatory filings and any related restrictions, limitations or warnings in the label of any approved product candidates;

- the significant control or influence that EA Pharma has over the commercialization of elobixibat in Japan and the development and commercialization of elobixibat in EA Pharma's other licensed territories;

- whether we elect to seek and, if so, our ability to establish a license or other partnering transaction with a third party for elobixibat in the United States or Europe;

- whether findings from nonclinical studies and clinical trials of IBAT inhibitors will be predictive of future clinical success for a product candidate of ours in the treatment of NASH;

- the accuracy of our estimates regarding expenses, costs, future revenues, uses of cash and capital requirements;

- our ability to obtain additional financing on reasonable terms, or at all;

- our ability to establish additional licensing, collaboration or similar arrangements on favorable terms and our ability to attract collaborators with development, regulatory and commercialization expertise;
- the success of competing third-party products or product candidates;
- our ability to successfully commercialize any approved product candidates, including their rate and degree of market acceptance;
- our ability to expand and protect our intellectual property estate;
- regulatory developments in the United States and other countries;
- our ability to fully remediate our identified internal control material weaknesses;
- the performance of our third-party suppliers, manufacturers and contract research organizations and our ability to obtain alternative sources of raw materials;
- our ability to attract and retain key personnel; and
- our ability to comply with regulatory requirements relating to our business, and the costs of compliance with those requirements, including those on data privacy and security.

These and other risks and uncertainties are described in greater detail under the caption "Risk Factors" in Item 1A of Part I of our Annual Report on Form 10-K for the fiscal year ended December 31, 2018, in Item 1A of Part II of this quarterly report, and in other filings that we make with the Securities and Exchange Commission, or SEC. As a result of the risks and uncertainties, the results or events indicated by the forward-looking statements may not occur. We caution you not to place undue reliance on any forward-looking statement.

In addition, any forward-looking statement in this quarterly report represents our views only as of the filing date of this quarterly report and should not be relied upon as representing our views as of any subsequent date. We anticipate that subsequent events and developments may cause our views to change. Although we may elect to update these forward-looking statements publicly at some point in the future, we specifically disclaim any obligation to do so, except as required by applicable law. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

Albireo Pharma, Inc.

Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

(unaudited)

	June 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 157,722	$ 163,885
Prepaid expenses and other current assets	5,310	3,765
Total current assets	163,032	167,650
Property and equipment, net	544	187
Goodwill	17,260	17,260
Other assets	1,115	369
Total assets	$ 181,951	$ 185,466
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payables	$ 3,837	$ 4,352
Accrued expenses	7,555	8,165
Other current liabilities	393	308
Total current liabilities	11,785	12,825
Liability related to sale of future royalties	52,224	49,969
Other long-term liabilities	255	35
Total liabilities	64,264	62,829
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at June 30, 2019 and December 31, 2018; 12,685,326 and 11,969,928 issued and outstanding at June 30, 2019 and December 31, 2018	126	120
Additional paid in capital	240,734	214,694
Accumulated other comprehensive income	6,582	4,293
Accumulated deficit	(129,755)	(96,470)
Total stockholders' equity	117,687	122,637
Total liabilities and stockholders' equity	$ 181,951	$ 185,466

See accompanying notes to Condensed Consolidated Financial Statements.

Albireo Pharma, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except share and per share data)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Revenue	$ 1,250	$ 730	$ 1,820	$ 11,932
Operating expenses:				
Research and development	11,034	6,411	19,363	12,562
General and administrative	5,485	4,238	10,778	8,366
Other operating expense, net	8	487	2,304	1,991
Total operating expenses	16,527	11,136	32,445	22,919
Operating loss	(15,277)	(10,406)	(30,625)	(10,987)
Interest expense, net	(1,351)	(1,666)	(2,660)	(2,682)
Non-operating expense, net	—	(2,531)	—	(2,553)
Net loss	$ (16,628)	$ (14,603)	$ (33,285)	$ (16,222)
Net loss per share attributable to holders of common stock:				
Net loss per common share - basic and diluted	$ (1.35)	$ (1.22)	$ (2.73)	$ (1.42)
Weighted-average common shares used to compute basic and diluted net loss per common share	12,355,969	11,938,357	12,178,376	11,417,463

See accompanying notes to Condensed Consolidated Financial Statements.

Albireo Pharma, Inc.

Condensed Consolidated Statements of Comprehensive Loss

(in thousands)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Net loss	$ (16,628)	$ (14,603)	$ (33,285)	$ (16,222)
Other comprehensive loss:				
Foreign currency translation adjustment	(9)	2,573	2,289	3,767
Total other comprehensive (loss) income	(9)	2,573	2,289	3,767
Total comprehensive loss	$ (16,637)	$ (12,030)	$ (30,996)	$ (12,455)

See accompanying notes to Condensed Consolidated Financial Statements.

Albireo Pharma, Inc.

Condensed Consolidated Statements of Stockholders' Equity

(in thousands, except share and per share data)

(unaudited)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income	Deficit	Equity
Balance--December 31, 2018	11,969,928	$ 120	$214,694	$ 4,293	$ (96,470)	$ 122,637
Stock-based compensation expense	—	—	1,823	—	—	1,823
Exercise of options	68,908	—	1,290	—	—	1,290
Other comprehensive income	—	—	—	2,298	—	2,298
Net loss	—	—	—	—	(16,657)	(16,657)
Balance--March 31, 2019	12,038,836	$ 120	$217,807	$ 6,591	$ (113,127)	$ 111,391
Stock-based compensation expense	—	—	2,049	—	—	2,049
Exercise of awards	9,123	—	110	—	—	110
Issuance of common stock, net of costs	637,367	6	20,768	—	—	20,774
Other comprehensive loss	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	(16,628)	(16,628)
Balance--June 30, 2019	12,685,326	$ 126	$240,734	$ 6,582	$ (129,755)	$ 117,687

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income	Deficit	Equity
Balance--December 31, 2017	8,902,784	$ 89	$114,522	$ 1,001	$ (50,359)	$ 65,253
Stock-based compensation expense	—	—	1,188	—	—	1,188
Issuance of common stock, net of costs	2,994,362	30	94,120	—	—	94,150
Other comprehensive income	—	—	—	1,194	—	1,194
Net loss	—	—	—	—	(1,619)	(1,619)
Balance--March 31, 2018	11,897,146	$ 119	$209,830	$ 2,195	$ (51,978)	$ 160,166
Stock-based compensation expense	—	—	1,056	—	—	1,056
Exercise of options	60,345	1	254	—	—	255
Other comprehensive income	—	—	—	2,573	—	2,573
Net loss	—	—	—	—	(14,603)	(14,603)
Balance--June 30, 2018	11,957,491	$ 120	$211,140	$ 4,768	$ (66,581)	$ 149,447

Albireo Pharma, Inc.

Condensed Consolidated Statements of Cash Flows

(in thousands)

(unaudited)

	Six Months Ended June 30,	
	2019	**2018**
Cash flows from operating activities:		
Net loss	$ (33,285)	$ (16,222)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non cash interest expense on liability related to royalty monetization	4,075	2,028
Depreciation and amortization	50	22
Stock-based compensation expense	3,872	2,244
Unrealized foreign exchange loss	3,508	6,718
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(1,662)	(1,377)
Other assets	(414)	354
Accounts payables	(393)	1,867
Accrued expenses	(2,272)	(2,223)
Other current and long-term liabilities	8	70
Net cash used in operating activities	(26,513)	(6,519)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(409)	(61)
Net cash used in investing activities	(409)	(61)
Cash flows from financing activities:		
Proceeds from issuance of common stock, net of issuance costs	20,774	94,149
Royalty monetization	—	44,525
Proceeds from exercise of options	1,400	255
Net cash provided by financing activities	22,174	138,929
Effect of exchange rate changes on cash and cash equivalents	(1,415)	(2,352)
Net (decrease) increase in cash and cash equivalents	(6,163)	129,997
Cash and cash equivalents—beginning of period	163,885	53,231
Cash and cash equivalents—end of period	$ 157,722	$ 183,228

See accompanying notes to Condensed Consolidated Financial Statements.

1. Summary of significant accounting policies and basis of presentation

Organization

Albireo Pharma, Inc. (Parent), together with its direct and indirect subsidiaries (the Company), is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel bile acid modulators to treat orphan pediatric liver diseases and other liver and gastrointestinal diseases and disorders. The Company's clinical pipeline includes a Phase 3 lead product, a Phase 2 product candidate, and elobixibat, which is approved in Japan for the treatment of chronic constipation. Odevixibat, the Company's Phase 3 lead product, is in development as a once per-day treatment given orally in a capsule or sprinkled over food, initially being evaluated using the planned commercial formulation in patients with progressive familial intrahepatic cholestasis (PFIC) types 1 and 2. PFIC is a rare, life-threatening genetic disorder affecting young children.

Basis of presentation

The accompanying unaudited Condensed Consolidated Financial Statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial information, and the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements and should be read in conjunction with the audited consolidated financial statements and accompanying notes included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018. The Company combined prepaid expenses and other assets with Other receivables and reflected this in the Condensed Consolidated Balance Sheets and the Condensed Consolidated Statements of Cashflows at June 2019 and December 2018, and for the six months ended June 2019 and 2018 respectively, with a change in the prior period presentation being made to conform to the current period presentation. There was no change to previously reported net loss or total comprehensive loss in the prior period presented as a result. In the opinion of management, all adjustments (including normal recurring accruals) considered necessary for fair presentation have been included in the Condensed Consolidated Financial Statements. The results of operations for the six months ended June 30, 2019 are not necessarily indicative of the results that may be expected for the full fiscal year, any other interim period or any future fiscal year. The condensed consolidated financial statements are prepared on a basis consistent with prior periods except for the adoption of the new leasing standard discussed below.

Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

Principles of consolidation

The accompanying Consolidated Financial Statements include the accounts of Parent and its direct or indirect wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Foreign currency translation

Functional currency

Items included in the financial statements of each entity comprising the Company are measured using the currency of the primary economic environment in which the entity operates (the functional currency).

Transactions and balances

Foreign currency transactions in each entity comprising the Company are remeasured into the functional currency of the entity using the exchange rates prevailing at the respective transaction dates. Foreign exchange gains and losses resulting from the settlement of such transactions and from the remeasurement at period-end exchange rates of monetary assets and liabilities denominated in foreign currencies are recognized within Other operating expense, net in the Condensed Consolidated Statements of Operations.

The results and financial position of the Company that have a functional currency different from the USD are translated into the presentation currency as follows:

a. assets and liabilities presented are translated at the closing exchange rate as of June 30, 2019 and December 31, 2018;

b. income and expenses for each statement of comprehensive loss are translated at the average exchange rate for the applicable period; and

c. significant transactions use the closing exchange rate on the date of the transaction;

All resulting exchange differences arising from such translations are recognized directly in other comprehensive income (loss) and presented as a separate component of equity.

Use of estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts of assets, liabilities, revenues and expenses reported in the financial statements and accompanying notes. Management must apply significant judgment in this process. On an ongoing basis, the Company evaluates its estimates and assumptions, including but not limited to accruals, deferred tax assets and the accretion of interest on the monetization liability. Actual results could materially differ from these estimates.

Revenue recognition

Milestone Payments

At the inception of each arrangement that includes development milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant revenue reversal would not occur. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

Royalties

In 2012, the Company entered into a license agreement (the Agreement) with EA Pharma Co., Ltd. (EA Pharma, formerly Ajinomoto Pharmaceuticals Co., Ltd.) to develop a select product candidate (elobixibat) for registration and subsequent commercialization in select markets. In conjunction with the Agreement, the Company granted EA Pharma an exclusive license to its intellectual property for development and commercialization activities in the designated field and territories. The Company is entitled to payments resulting from pharmaceutical ingredient or related procurement services if provided as part of a development plan. Revenue related to these payments is recorded on a net basis; in this

instance, the Company acts as an agent, as it does not have discretion to change suppliers and does not perform any part of the services or manufacture of the subject pharmaceutical ingredients. The costs associated with these activities are netted against the related revenue in the condensed consolidated statements of operations.

As of June 30, 2019, the Company is eligible to receive a regulatory-based milestone payment under the Agreement of €4.3 million ($4.9 million based on the Euro to USD exchange rate as of June 30, 2019) if a specified regulatory event is achieved for elobixibat. The cash payments and any other payments for milestones and royalties from EA Pharma are non-refundable, non-creditable and not subject to set-off.

In January 2018, the Japanese Ministry of Health Labour and Welfare (MHLW) approved a new drug application filed by EA Pharma for elobixibat for the treatment of chronic constipation, for which the Company received a milestone payment of $11.2 million. Based on the regulatory approval, the Company determined that the milestone was no longer at risk of significant reversal. As such, because the single performance obligation had previously been satisfied, the Company recognized this amount in full in the first quarter of 2018 and there was no deferred revenue or contract asset as of December 31, 2018. The Company recognizes the royalty revenue based on the estimated qualifying sales by EA Pharma each period.

Monetization of Future Royalties

In December 2017, the Company entered into a royalty interest acquisition agreement (RIAA) with HealthCare Royalty Partners III, L.P. (HCR) pursuant to which it sold to HCR the right to receive all royalties from sales in Japan and sales milestones achieved from any covered territory potentially payable to the Company under the Agreement, up to a specified maximum "cap" amount of $78.8 million, based on the funds the Company received from HCR to date. The Company received $44.5 million from HCR, net of certain transaction expenses, under the RIAA and the Company is eligible to receive an additional $15.0 million under the RIAA if a specified sales milestone is achieved for elobixibat in Japan. If the cap amount is reached, the Company will again become eligible to receive royalties from Japanese sales and sales milestones from covered territories for elobixibat from EA Pharma under the Agreement. The Company is obligated to make royalty interest payments to HCR under the RIAA only to the extent it receives future Japanese royalties, sales milestones or other specified payments from EA Pharma. Although the Company sold its rights to receive royalties from the sales of elobixibat in Japan, as a result of its ongoing involvement in the cash flows related to these royalties, the Company will continue to account for these royalties as revenue. The Company recorded the $44.5 million as a liability related to sale of future royalties (royalty obligation). The royalty obligation will be amortized using the effective interest rate method, based on the Company's best estimate of the time it will take to reach the capped amount.

The following table shows the activity within the liability account for the period ended June 30, 2019:

	June 30, 2019
	(in thousands)
Liability related to sale of future royalties—beginning balance	$ 50,546
Foreign currency translation gain	(19)
Accretion of interest expense on liability related to royalty monetization	4,075
Repayment of the liability	(1,128)
Liability related to sale of future royalties—ending balance	$ 53,474
Less current portion classified within accrued expenses	(1,250)
Net ending liability related to sale of future royalties	$ 52,224

The Company records estimated royalties due for the current period in accrued other expenses until the payment is received from EA Pharma at which time the Company then remits payment to HCR. As royalties are remitted to HCR, the balance of the royalty obligation will be effectively repaid over the life of the RIAA. In order to determine the amortization of the royalty obligation, the Company is required to estimate the total amount of future royalty payments to be received and submitted to HCR, as noted above, based on the Company's best estimate of the time it will take to reach the cap amount and when milestones will be received. The sum of these amounts less the $44.5 million proceeds the Company received will be recorded as interest expense over the life of the royalty obligation. Since inception, the Company's estimate of its total interest expense resulted in a quarterly effective interest rate of approximately 4.03%

The Company periodically assesses the estimated royalty payments to HCR and to the extent such payments are greater or less than its initial estimates or the timing of such payments is materially different than its original estimates, the Company will prospectively adjust the accretion of interest on the royalty obligation. There are a number of factors that could materially affect the amount and the timing of royalty payments, most of which are not within the Company's control. Such factors include, but are not limited to, the rate of elobixibat prescriptions, the number of doses administered, the introduction of competing products, manufacturing or other delays, patent protection, adverse events that result in governmental health authority imposed restrictions on the use of the drug products, significant changes in foreign exchange rates as the royalties remitted to HCR are in U.S. dollars while sales of elobixibat are in Japanese yen, and sales never achieving forecasted numbers, which would result in reduced royalty payments and reduced non-cash interest expense over the life of the royalty obligation. To the extent future royalties result in an amount less than the liability, the Company is not obligated to fund any such shortfall.

Recently adopted accounting pronouncements

As of January 1, 2019, the Company adopted ASU 2016-02, "*Leases (Topic 842)*." The new standard establishes a right-of-use (ROU) model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. The Company has applied the transition provisions at the beginning of the period of adoption, which results in recording the cumulative adjustment to the opening balance sheet as of January 1, 2019. Under this transition provision, the Company will continue to apply the legacy guidance under ASC 840, *Leases*, including its disclosure requirements, in the comparative periods presented in fiscal 2019. On the date of the adoption, the Company recorded a ROU asset of $1.2 million and lease liabilities of $1.2 million. Additionally, the Company elected the following practical expedients: the Company has elected to not separate lease components from non-lease components in its lease contract; the Company will not apply the recognition requirements of ASC 842 to its leases with lease terms of 12 months or less but rather recognize the lease expense on a straight-line basis over the lease term; *Relief package* – the Company has not reassessed whether expired or existing contracts may contain a lease, the lease classification of expired or existing leases and whether previously capitalized indirect costs would qualify for capitalization under ASC 842. *Use of hindsight* – the Company has elected to use hindsight in assessing the likelihood of renewals, terminations and purchase options and in assessing impairment of ROU assets. *Portfolio approach* – the Company has elected to not apply the portfolio approach for groups of leases with similar characteristics.

2. Fair Value of financial instruments

When measuring the fair value of financial instruments, the Company evaluates valuation techniques such as the market approach, the income approach and the cost approach. A three-level valuation hierarchy, which prioritizes the inputs to valuation techniques that are used to measure fair value, is based upon whether such inputs are observable or unobservable.

Observable inputs reflect market data obtained from independent sources, while unobservable inputs reflect market assumptions made by the reporting entity. The three-level hierarchy for the inputs to valuation techniques is briefly summarized as follows:

Level 1—Observable inputs such as quoted prices (unadjusted) for *identical* instruments in active markets;

Level 2—Observable inputs such as quoted prices for *similar* instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, or model-derived valuations whose significant inputs are observable for substantially the full term of the assets or liabilities; and

Level 3—Unobservable inputs that reflect the reporting entity's estimate of assumptions that market participants would use in pricing the asset or liability.

3. Commitments and contingencies

Commercial real estate leases

The Company's portfolio of commercial real estate leases consists of office space for its corporate headquarters in Boston, Massachusetts and for administrative and research lab space in Göteborg, Sweden, both of which are accounted for as operating leases. These leases include renewal rights and, as for the corporate headquarters lease, escalating payments. On March 28, 2019, the Company entered into an amendment to the Boston, Massachusetts lease to (i) replace the Company's prior office space with a new office space that is being leased from the same landlord and (ii) extend the term of the lease through the date ending eighty-eight months following July 1, 2019, when the Company took control of the new leased space. The new leased space contains monthly lease payments subject to annual escalations of $1.00 per square foot for the remaining term of the lease with the Company obligated to make approximately $7.3 million of aggregate lease payments over the term of the lease, or approximately $900,000 annually.

The Company's lease in Göteborg, Sweden includes the rental of office and lab space plus a defined number of parking spaces and contained an original expiration date in November 2019. This lease includes annual rent escalations based on the changes in the Swedish Consumer Price Index. This lease renews automatically for consecutive three year terms unless notice of non-renewal is given by either party at least nine months prior to the end of the current term and subject to the Company's right to terminate the lease at any time upon six months' notice. Subsequent to the year ended December 31, 2018, this lease was renewed for an additional three year period through November 2022, with quarterly payments of $35,419.

As of June 30, 2019, the net balance of ROU assets totaled $0.3 million and were classified within other non-current assets. The current and long term balances of lease liabilities at June 30, 2019 were $0.1 million and $0.2 million, respectively, and were classified within other liabilities, and long-term liabilities, respectively. Operating lease expense under ASC 842 was $0.1 million and $0.2 million respectively for the three months and six months ended June 30, 2019. There were no short-term lease or variable lease costs incurred for the six months ended June 30, 2019. As of June 30, 2019, the weighted average remaining lease term for the Company's operating leases was 3.4 years. Rent expense recognized under legacy GAAP for the Company's operating leases was $0.1 million and $0.2 million for the three and six months ended June 30, 2018, respectively.

Agreements with CROs

As of June 30, 2019, the Company had various agreements with CROs for the conduct of specified research and development activities. Based on the terms of the respective agreements, the Company may be required to make future payments of up to $24.7 million to CROs upon the completion of contracted work.

Legal Contingency

On February 19, 2019, the Company filed a complaint for breach of contract and breach of implied covenant of good faith and fair dealing against Ferring International Center S.A. (the Respondent) in the United States District Court for the Southern District of New York. Based on procedural considerations, we decided to refile the complaint in the Supreme Court of the State of New York, County of New York on April 26, 2019. We previously entered into the License Agreement, dated July 2, 2012, as amended as of October 2013 (the License Agreement), by and between Respondent and us, pursuant to which Respondent, among other things, conducted two Phase 3 clinical trials to evaluate the efficacy and safety of elobixibat as a treatment for chronic idiopathic constipation, known as Echo 1 and Echo 2, which ended in 2014. As previously disclosed, Respondent stopped Echo 1 and Echo 2 early citing an issue related to the distribution of study drug to study sites that was unrelated to the performance of elobixibat and terminated the License Agreement. The complaint alleges that Respondent breached its obligations under the License Agreement to (1) make earned milestone payments, (2) use good clinical practices, good laboratory practices and good manufacturing practices, and (3) use commercially reasonable efforts. The complaint also alleges that Respondent violated the covenant of good faith and fair dealing implied in the License Agreement. In the complaint, the Company is seeking, among other things, compensatory damages of at least € 37 million (converted to $42.2 million as of June 30, 2019). On July 31, 2019 Respondent filed a motion to dismiss the complaint.

The Company has retained outside counsel under a contingency fee arrangement, and as a result, the Company will not incur attorneys' fees for litigating the matter, but counsel will receive a contingent fee of 33 1/3% of the net recovery (after deduction of expenses) in the event a recovery is received.

Due to their nature, it is difficult to predict the outcome, or the costs involved in any litigation. Furthermore, Respondent may have significant resources and interest to litigate and therefore, although we have a contingency fee arrangement, this litigation could be protracted and may ultimately involve significant legal expenses.

4. Net loss per share

Basic net loss per share, or Basic EPS, is calculated by dividing the net loss by the weighted average number of shares of common stock outstanding. Diluted net loss per share, or Diluted EPS, is calculated by dividing the net loss by the weighted-average number of shares of common stock plus dilutive common stock equivalents outstanding.

The following table sets forth the computation of Basic EPS and Diluted EPS (in thousands, except for share and per share data):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Basic and Diluted EPS:				
Numerator				
Net loss	$ (16,628)	$ (14,603)	$ (33,285)	$ (16,222)
Denominator				
Weighted average number of shares outstanding	12,355,969	11,938,357	12,178,376	11,417,463
Basic and Diluted EPS	$ (1.35)	$ (1.22)	$ (2.73)	$ (1.42)

The following outstanding common stock equivalents were excluded from the computation of Diluted EPS for the three and six months ended March 31, 2019 and 2018 because including them would have been anti-dilutive:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2019	2018	2019	2018
Options to purchase common stock and RSUs	1,820,351	1,393,297	1,820,351	1,393,297

5. Income taxes

The Company did not record a tax provision or benefit for the three months ended June 30, 2019 or 2018. The Company has continued to maintain a full valuation allowance against its net deferred tax assets. The Company has had an overall net operating loss position since its inception.

6. Financings

At-the-Market Offering Program

In October 2017, the Company entered into an at-the-market offering program, which we refer to as the 2017 Sales Agreement relating to the sale of shares of the Company's common stock having an aggregate offering price of up to $50.0 million. In February 2018, the Company sold an aggregate of 728,862 shares of common stock pursuant to the 2017 Sales Agreement and received proceeds, net of offering expenses, of approximately $24.2 million. On March 6, 2019, the Company terminated the 2017 Sales Agreement and entered into a new sales agreement, which we refer to as the 2019 Sales Agreement, with respect to an at-the-market offering program relating to the sale of shares of the Company's common stock having an aggregate offering price of up to $50.0 million. In May 2019, the Company sold an aggregate of 637,367 shares of common stock pursuant to the 2019 Sales Agreement and received proceeds, net of offering expenses, of approximately $20.8 million.

January 2018 Underwritten Public Offering

On January 9, 2018, the Company completed an underwritten public offering of 2,265,500 shares of its common stock, at a price to public of $33.00 per share. The Company received net proceeds from this offering of $69.9 million, after deducting underwriting discounts, commission and offering expenses.

7. Stock-based Compensation

The Company granted 482,961 options at a weighted average price of $25.63 and 52,000 RSUs with a weighted average grant date fair value of $26.31 during the six months ended June 30, 2019.

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
	(in thousands)		(in thousands)	
Employee awards:				
Research and development expense	$ 792	$ 666	$ 1,500	$ 1,534
General and administrative expense	1,257	390	2,372	710
Total stock-based compensation expense	$ 2,049	$ 1,056	$ 3,872	$ 2,244

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

You should read the following discussion and analysis of our financial condition and results of operations together with our condensed consolidated financial statements and the related notes included elsewhere in this quarterly report and our audited financial statements and Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K for the year ended December 31, 2018, filed with the SEC. In addition to historical information, the following discussion contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results, performance or experience could differ materially from what is indicated by any forward-looking statement due to various important factors, risks and uncertainties, including, but not limited to, those set forth under "Cautionary Note Regarding Forward-Looking Statements" included elsewhere in this quarterly report or under "Risk Factors" in Item 1A of Part I of our Annual Report on Form 10-K for the year ended December 31, 2018, in Item 1A of Part II of this Quarterly Report on Form 10-Q, or in other filings that we make with the SEC.

Overview

We are a biopharmaceutical company focused on the development and commercialization of novel bile acid modulators to treat orphan pediatric liver diseases and other liver or gastrointestinal diseases and disorders. The initial target indication for our lead product candidate, odevixibat, is progressive familial intrahepatic cholestasis, or PFIC, a rare, life-threatening genetic disorder affecting young children for which there is currently no approved drug treatment. We completed a Phase 2 clinical trial of odevixibat in children with chronic cholestasis and pruritus, and in May of 2018 we enrolled the first patient in our Phase 3 clinical trial for odevixibat, given once per day as an oral capsule or sprinkled over food, in patients with PFIC types 1 and 2, which we refer to as PEDFIC 1. We are using the planned commercial formulation in PEDFIC 1, but any commercial product will include final trade dress. In the first quarter 2019, we revised our statistical analysis methodology for PEDFIC 1, in line with guidance from the FDA. One result of the revision is an improvement in the power of the study. We expect to have top line data from PEDFIC 1 in mid-2020. We also submitted a protocol amendment for PEDFIC 2, our long term, open label extension study, which includes an additional cohort of PFIC patients who are not eligible for PEDFIC 1. The first sites have been activated for the expanded PEDFIC 2 cohort. In June of 2018, the FDA granted a rare pediatric disease designation to odevixibat for the treatment of PFIC, which affirms our eligibility to apply for a rare pediatric disease priority review voucher upon submission of a new drug application for odevixibat. In September of 2018, the FDA granted fast track designation for odevixibat for the treatment of pruritus associated with PFIC. In October of 2018, the FDA granted orphan drug designation to odevixibat for the treatment of Alagille syndrome, or ALGS, a rare, life threatening disease that affects the liver and for which there is no approved pharmacologic treatment option. In December of 2018, the European Commission granted orphan designation to odevixibat for the treatment of biliary atresia, another rare, life threatening disease that affects the liver and for which there is no approved pharmacologic treatment option. In January of 2019, the FDA granted orphan drug designation to odevixibat for the treatment of biliary atresia. In addition to PFIC, we plan to initiate a pivotal clinical trial for odevixibat in biliary atresia, which we believe to be one of the most common rare pediatric liver diseases, in 2020, and we plan to conduct clinical development of odevixibat in 2020 as a treatment for one or more other pediatric cholestatic liver diseases and disorders. Our most advanced product candidates in addition to odevixibat include elobixibat, which is approved in Japan for the treatment of chronic constipation and for which we initiated a Phase 2 clinical trial as a treatment for nonalcoholic fatty liver disease, or NAFLD, and NASH, with the first patients enrolled in June 2019, and A3384, which is a product candidate to treat bile acid malabsorption, or BAM. We have method of use patents for odevixibat with a natural expiry in 2031, but which can run through 2034 with potential patent term extensions. In June 2018, we were granted a patent for a method of using elobixibat to treat NASH in both the U.S. and Europe. We also have a preclinical program in NASH.

The precise prevalence of PFIC is unknown, and we are not aware of any patient registries or other method of establishing with precision the actual number of patients with PFIC in any geography. PFIC has been estimated to affect between one in every 50,000 to 100,000 children born worldwide. Benign recurrent familial intrahepatic cholestasis, or BRIC, is a disease that is caused by the same genetic defect as PFIC, and patients who manifest the same symptoms as PFIC but their symptomatology tends to be episodic in nature. We estimate that BRIC affects between one in every 50,000 to 100,000 children born worldwide. Based on the published incidence, published regional populations, and

estimated median life expectancies, we estimate the prevalence of PFIC together with BRIC to be approximately 8,000 to 10,000 patients in the U.S. and E.U. but we are not able to estimate the prevalence of PFIC or BRIC with precision.

We estimate that there are approximately 3,000 to 4,000 PFIC patients in the U.S. and E.U. We also estimate that there are approximately 5,000 to 6,000 BRIC patients in the U.S. and E.U. We currently have not modeled other regional opportunities in Asia, the Middle East and Latin America. We are aware there may be higher prevalence of disease in some countries such as Saudi Arabia and Turkey. We hold global rights to odevixibat unencumbered. Our current plan is to commercialize odevixibat ourselves in the U.S. and E.U., and we have begun the process of identifying potential partners for other regions. There are currently no drugs approved for the treatment of PFIC. First-line treatment for PFIC is typically off-label ursodeoxycholic acid, or UDCA, which is approved in the United States and elsewhere for the treatment of primary biliary cholangitis, or PBC. However, many PFIC patients do not respond well to UDCA, undergo partial external bile diversion, or PEBD, surgery and often require liver transplantation. PEBD surgery is a life-altering and undesirable procedure in which bile is drained outside the body to a stoma bag that must be worn by the patient 24 hours a day.

Other Indications Under Development for Odevixibat. We plan to initiate a pivotal clinical trial with odevixibat in biliary atresia in 2020. We plan to conduct clinical development of odevixibat in 2020 as a treatment for other pediatric cholestatic liver diseases and disorders as well, which may include ALGS and primary sclerosing cholangitis.

Biliary atresia is a partial or total blocking or absence of large bile ducts that causes cholestasis and resulting accumulation of bile that damages the liver. The estimated worldwide incidence of biliary atresia is between 4.5 and 8.5 for every 100,000 live births. There are currently no drugs approved for the treatment of biliary atresia. The current standard of care is a surgery known as the Kasai procedure, or hepatoportoenterostomy, in which the obstructed bile ducts are removed and a section of the small intestine is connected to the liver directly. However, only an estimated 25% of those initially undergoing the Kasai procedure will survive to their twenties without need for liver transplantation. The European Commission granted orphan designation to odevixibat for the treatment of biliary atresia in December of 2018. In January of 2019, the FDA granted orphan drug designation to odevixibat for the treatment of biliary atresia. We intend to initiate a pivotal clinical trial with odevixibat for the treatment of biliary atresia in 2020.

ALGS is a genetic condition associated with liver, heart, eye, kidney and skeletal abnormalities. In particular, ALGS patients have fewer than normal bile ducts inside the liver, which leads to cholestasis and the accumulation of bile and causes scarring in the liver. ALGS is estimated to affect between one in every 30,000 to 70,000 children born worldwide. There are currently no drugs approved for the treatment of ALGS. Current treatment for ALGS is generally in line with current treatments for PFIC as described above. In October of 2018, the FDA granted orphan drug designation to odevixibat for the treatment of ALGS.

Primary sclerosing cholangitis refers to swelling (inflammation), scarring, and destruction of bile ducts inside and outside of the liver. The first symptoms are typically fatigue, itching and jaundice, and many patients with sclerosing cholangitis also suffer from inflammatory bowel disease. The estimated incidence of primary sclerosing cholangitis is 6.3 cases per 100,000 people. There are currently no drugs approved for the treatment of sclerosing cholangitis. First-line treatment is typically off-label UDCA, although UDCA has not been established to be safe and effective in patients with sclerosing cholangitis in well controlled clinical trials.

Elobixibat as a potential treatment for NASH. NASH is a common, serious and sometimes fatal chronic liver disease that resembles alcoholic liver disease but occurs in people who drink little or no alcohol. Based on multiple epidemiological studies published by third parties in 2014 and 2015, we estimate that NASH affects 2 to 3.5% of adults, representing over 9 million people in the United States and 10 million people in the European Union. There are currently no drugs approved for the treatment of NASH. Lifestyle changes, including modification of diet and exercise to reduce body weight, as well as treatment of concomitant diabetes and dyslipidemia, are commonly accepted as the standard of care for NASH, but have not conclusively been shown to prevent disease progression. Based on findings on parameters relevant to NASH in clinical trials of elobixibat that we previously conducted in patients with chronic constipation and in patients with elevated cholesterol and findings on other parameters relevant to NASH from nonclinical studies that we previously conducted with elobixibat or a different IBAT inhibitor, we believe elobixibat has potential benefit in the treatment of NASH. We initiated our Phase 2 clinical trial of elobixibat in NAFLD and NASH, with the first patients enrolled in June2019.

Since inception, we have incurred significant operating losses. As of June 30, 2019, we had an accumulated deficit of $129.8 million. We expect to continue to incur significant expenses and increasing operating losses as we continue our development of, and seek marketing approvals for, our product candidates, prepare for and begin the commercialization of any approved products, and add infrastructure and personnel to support our product development efforts and operations as a public company in the United States.

As a clinical-stage company, our revenues, expenses and results of operations are likely to fluctuate significantly from quarter to quarter and year to year. We believe that period-to-period comparisons of our results of operations should not be relied upon as indicative of our future performance.

As of June 30, 2019, we had approximately $157.7 million in cash and cash equivalents.

Financial Operations Overview

The following discussion sets forth certain components of our consolidated statements of operations as well as factors that impact those items.

Revenue

We generate revenue primarily from the receipt of royalty revenue, upfront or license fees and milestone payments. License agreements with commercial partners generally include nonrefundable upfront fees and milestone payments, the receipt of which is dependent upon the achievement of specified development, regulatory or commercial milestone events, as well as royalties on product sales of licensed products, if and when such product sales occur, and payments for pharmaceutical ingredient or related procurement services. For these agreements, management applies judgment in the allocation of total agreement consideration to the performance obligations on a reliable basis that reasonably reflects the selling prices that might be expected to be achieved in stand-alone transactions. For additional information about our revenue recognition, refer to Note 1 to our condensed consolidated financial statements included in this quarterly report.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of personnel costs (including salaries, benefits and stock-based compensation) for employees in research and development functions, costs associated with nonclinical and clinical development services, including clinical trials and related manufacturing costs, third-party contract research organizations, or CROs, and related services and other outside costs, including fees for third-party professional services such as consultants. Our nonclinical studies and clinical studies are performed by CROs. We expect to continue to focus our research and development efforts on nonclinical studies and clinical trials of our product candidates. As a result, we expect our research and development expenses to continue to increase for the foreseeable future.

Our direct research and development expenses are tracked on a program-by-program basis and consist primarily of external costs such as fees paid to CROs and others in connection with our nonclinical and clinical development activities and related manufacturing. We do not allocate employee costs or facility expenses, including depreciation or other indirect costs, to specific product development programs because these costs are deployed across multiple product development programs and, as such, are not separately classified.

Successful development of our current and potential future product candidates is highly uncertain. Completion dates and costs for our programs can vary significantly by product candidate and are difficult to predict. As a result, we cannot estimate with any degree of certainty the costs we will incur in connection with development of any of our product candidates. We anticipate we will make determinations as to which programs and product candidates to pursue and how much funding to direct to each program and product candidate on an ongoing basis in response to the results of ongoing and future clinical trials, our ability to enter into licensing, collaboration and similar arrangements with respect to current or potential future product candidates, the success of research and development programs and our assessments of commercial potential.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs (including salaries, benefits and stock-based compensation) for our executive, finance and other administrative employees. In addition, general and administrative expenses include fees for third-party professional services, including consulting, information technology, legal and accounting services and other corporate expenses and allocated overhead.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our unaudited condensed consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles for interim financial information. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses. We base our estimates and assumptions on historical experience and on various assumptions that we believe are reasonable under the circumstances, and we evaluate them on an ongoing basis. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities and the recording of revenues and expenses that are not readily apparent from other sources. Actual results and experiences may differ materially from these estimates and judgments. In addition, our reported financial condition and results of operations could vary if new accounting standards are enacted that are applicable to our business.

Our critical accounting policies and the methodologies and assumptions we apply under them have not materially changed since March 6, 2019, the date we filed our Annual Report on Form 10-K for the year ended December 31, 2018. For more information on our critical accounting policies, refer to our Annual Report on Form 10-K for the year ended December 31, 2018.

Results of Operations

Three Months Ended June 30, 2019 and June 30, 2018

Result of Operations

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Revenue	$ 1,250	$ 730	$ 520
Operating Expenses			
Research and development	11,034	6,411	4,623
General and Administrative	5,485	4,238	1,247
Other operating expense, net	8	487	(479)
Total operating expenses	16,527	11,136	5,391
Operating loss	(15,277)	(10,406)	(4,871)
Interest expense, net	(1,351)	(1,666)	315
Non-operating expense, net	-	(2,531)	2,531
Net loss	$ (16,628)	$ (14,603)	$ (2,025)

Revenue

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Revenue	$ 1,250	$ 730	$ 520

There was $1.3 million in revenue for the three months ended June 30, 2019 compared with $0.7 million for the three months ended June 30, 2018, an increase of $0.5 million. The higher revenue is due to the estimated royalty revenue received from EA Pharma for elobixibat for the treatment of chronic constipation.

Research and development expenses

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Research and development expenses	$ 11,034	$ 6,411	$ 4,623

Research and development expenses were $11.0 million for the three months ended June 30, 2019 compared with $6.4 million for the three months ended June 30, 2018, an increase of $4.6 million. The higher research and development expenses for the 2019 period were principally due to personnel expenses, and program expenses as we continue to increase our headcount, and program activities, respectively.

The following table summarizes our principal product development programs and the out-of-pocket third-party expenses incurred with respect to each clinical-stage product candidate and our preclinical programs for the three months ended June 30, 2019 and 2018.

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Direct third-party project costs:			
Odevixibat	$ 4,469	$ 3,461	$ 1,008
Elobixibat	1,148	72	1,076
A3384	151	205	(54)
Preclinical	1,317	462	855
Total	$ 7,085	$ 4,200	$ 2,885
Other project costs[1]:			
Personnel costs	$ 2,770	$ 1,347	$ 1,423
Other costs[2]	1,179	864	315
Total	$ 3,949	$ 2,211	$ 1,738
Total research and development costs	$ 11,034	$ 6,411	$ 4,623

(1) Other project costs are leveraged across multiple programs.
(2) Other costs include facility, supply, consultant and overhead costs that support multiple programs.

General and administrative expenses

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
General and administrative expenses	$ 5,485	$ 4,238	$ 1,247

General and administrative expenses were $5.5 million for the three months ended June 30, 2019 compared with $4.2 million for the three months ended June 30, 2018, an increase of $1.2 million. The increase is primarily attributable to personnel and related expenses as we continue to increase our headcount.

Other operating expense, net

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Other operating expense, net	$ 8	$ 487	$ (479)

Other operating expense, net totaled $0.0 million for the three months ended June 30, 2019 compared with $0.5 million for the three months ended June 30, 2018. The difference resulted primarily from changes in currency exchange rates in the two periods.

Interest expense, net

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Interest expense, net	$ (1,351)	$ (1,666)	$ 315

Interest expense, net totaled $1.4 million of expense for the three months ended June 30, 2019 compared with $1.7 million of expense for the three months ended June 30, 2018. The difference was principally attributable to interest income associated with our interest bearing cash and cash equivalents offset by non-cash interest expense recorded in connection with the sale of future royalties, related to sales of elobixibat in Japan.

Other non-operating expense, net

	Three Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Other non-operating expense, net	$ —	$ (2,531)	$ 2,531

Other non-operating expense, net for the three months ended June 30, 2018 was $2.5 million primarily related to the foreign currency expense associated with our royalty monetization in 2018. There was no other non-operating expense, net for the three months ended June 30, 2019.

Six Months Ended June 30, 2019 and June 30, 2018

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Revenue	$ 1,820	$ 11,932	$ (10,112)
Operating Expenses			
Research and development	19,363	12,562	6,801
General and Administrative	10,778	8,366	2,412
Other operating expense, net	2,304	1,991	313
Total operating expenses	32,445	22,919	9,526
Operating loss	(30,625)	(10,987)	(19,638)
Interest expense, net	(2,660)	(2,682)	22
Non-operating expense, net	-	(2,553)	2,553
Net loss	$ (33,285)	$ (16,222)	$ (17,063)

Revenue

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Revenue	$ 1,820	$ 11,932	$ (10,112)

There was $1.8 million in revenue for the six months ended June 30, 2019 compared with $11.9 million for the six months ended June 30, 2018, a decrease of $10.1 million. The decrease in revenue is due to a milestone payment received in the second quarter of 2018 from EA Pharma due to the approval by the Japanese MHLW of the drug application for elobixibat for the treatment of chronic constipation and the estimated royalty revenue from EA Pharma for elobixibat for the period.

Research and development expenses

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Research and development expenses	$ 19,363	$ 12,562	$ 6,801

There was $19.4 million in research and development expenses for the six months ended June 30, 2019 compared with $12.6 million for the six months ended June 30, 2018, an increase of $6.8 million. The higher research and development expenses for the 2019 period were principally due to personnel expenses, and program expenses as we continue to increase our headcount, and program activities, respectively.

The following table summarizes our principal product development programs and the out-of-pocket third-party expenses incurred with respect to each clinical-stage product candidate and our preclinical programs for the six months ended June 30, 2019 and 2018.

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Direct third-party project costs:			
Odevixibat	$ 7,833	$ 6,851	$ 982
Elobixibat	1,371	95	1,276
A3384	225	333	(108)
Preclinical	2,320	800	1,520
Total	$ 11,749	$ 8,079	$ 3,670
Other project costs[1]:			
Personnel costs	$ 5,470	$ 2,757	$ 2,713
Other costs[2]	2,144	1,726	418
Total	$ 7,614	$ 4,483	$ 3,131
Total research and development costs	$ 19,363	$ 12,562	$ 6,801

(1) Other project costs are leveraged across multiple programs.
(2) Other costs include facility, supply, consultant and overhead costs that support multiple programs.

General and administrative expenses

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
General and administrative expenses	$ 10,778	$ 8,366	$ 2,412

There was $10.8 million in general and administrative expenses for the six month ended June 30, 2019 compared with $8.4 million for the six months ended June 30, 2018, an increase of $2.4 million. The increase is primarily attributable to personnel and related expenses as we continue to increase our headcount.

Other operating expense, net

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Other operating expense, net	$ 2,304	$ 1,991	$ 313

Other operating expense, net totaled $2.3 million for the six months ended June 30, 2019 compared with $2.0 million for the six months ended June 30, 2018. The difference resulted primarily from changes in currency exchange rates in the two periods.

Interest expense, net

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Interest expense, net	$ (2,660)	$ (2,682)	$ 22

Interest expense, net totaled $2.7 million of expense for the six months ended June 30, 2019 compared with $2.7 million of expense for the six months ended June 30, 2018. The difference was principally attributable to non-cash interest expense recorded in connection with the sale of future royalties, related to sales of elobixibat in Japan, offset by interest income.

Other non-operating expense, net

	Six Months Ended June 30,		Change
	2019	2018	$
	(in thousands)		
Other non-operating expense, net	$ —	$ (2,553)	$ 2,553

Other non-operating expense, net for the six months ended June 30, 2018 was $2.6 million primarily related to the foreign currency expense associated with our royalty monetization in 2018. There was no other non-operating expense, net for the six months ended June 30, 2019.

Liquidity and Capital Resources

Sources of Liquidity

We do not expect to generate significant revenue from product sales unless and until we or a potential future licensee or collaborator obtains marketing approval for, and commercializes, one or more of our current or potential future product candidates (other than elobixibat as a treatment for chronic constipation in Japan), which we do not expect to occur until at least 2021, if at all. We anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of and seek regulatory approvals for our product candidates. We are subject to all of the risks applicable to the development of new pharmaceutical products and may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may harm our business. We expect that we will need substantial additional funding to complete development of and potentially commercialize our product candidates.

Our operations have historically been financed primarily through issuances of equity or convertible debt, upfront fees paid upon entering into license agreements, payments received upon the achievement of specified milestone events under license agreements, grants and venture debt borrowings. Our primary uses of capital are, and we expect will continue to be, personnel-related costs, third party expenses associated with our research and development programs, including the conduct of clinical trials, and manufacturing-related costs for our product candidates.

As of June 30, 2019, our cash and cash equivalents were approximately $157.7 million.

During the first quarter of 2018, following the Japanese MHLW's approval of elobixibat for the treatment of chronic constipation in January 2018, we received a $44.5 million payment, net of certain transaction expenses, from HCR under our RIAA. Under the terms of the RIAA, we are eligible to receive an additional $15 million if a specified sales

milestone is achieved for elobixibat in Japan. Additionally, this approval triggered a milestone payment to us from EA Pharma of $11.2 million. As of June 30, 2019, we have received approximately $45.4 million in upfront and milestone payments from EA Pharma under a license agreement for the development and commercialization of elobixibat in specified countries in Asia. We are eligible to receive additional amounts of up to $4.9 million under the amended agreement, if a specified regulatory event is achieved for elobixibat. In addition, subject to the terms of the RIAA with HCR, we may in the future also become eligible under the license agreement to receive up to $31.9 million, if specified sales milestones are achieved for elobixibat and stepped royalties at rates beginning in the high single digits on any future elobixibat product sales.

In January 2018, we completed an underwritten public offering of 2,265,500 shares of our common stock for net proceeds of approximately $69.9 million. Subsequently, in February 2018, we sold 728,862 shares of our common stock for net proceeds of approximately $24.2 million pursuant to an at-the-market offering program Sales Agreement that we entered into with Cowen in October 2017, or the 2017 Sales Agreement. This agreement terminated on March 6, 2019. These sales were registered on our universal shelf registration statement on Form S-3, which was declared effective on December 5, 2017, or the 2017 Form S-3.

On March 6, 2019, we filed a new universal shelf registration on Form S-3 with the SEC, which was declared effective on April 30, 2019, pursuant to which we registered for sale up to $200 million of any combination of our common stock, preferred stock, debt securities, warrants, rights and/or units from time to time and at prices and on terms that we may determine, which we refer to as the 2019 Form S-3.

On March 6, 2019, we entered into a new sales agreement, which we refer to as the 2019 Sales Agreement, with respect to an at-the-market offering program under which we may offer and sell, from time to time at our sole discretion, shares of our common stock having an aggregate offering price of up to $50.0 million. Subsequently, in May 2019, we sold 637,367 shares of our common stock for net proceeds of approximately $20.8 million pursuant to the 2019 Sales Agreement.

Cash Flows

Six Months Ended June 30, 2019 and June 30, 2018

	Six Months Ended June 30,	
	2019	2018
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ (26,513)	(6,519)
Investing activities	(409)	(61)
Financing activities	22,174	138,929
Total	$ (4,748)	$ 132,349
Effect of exchange rate changes on cash and cash equivalents	(1,415)	(2,352)
Net increase (decrease) in cash and cash equivalents	(6,163)	129,997

Operating activities

Cash used in operating activities of $26.5 million during the six months ended June 30, 2019 was primarily a result of our $33.3 million net loss from operations and a net decrease in assets and liabilities of $4.7 million. The net decrease in operating assets and liabilities during the six months ended June 30, 2019 was primarily driven by decreases in accounts payable, accrued expenses and an increase to prepaid expenses and other current assets, and other assets. This decrease was offset by non cash items, including$4.1 million of non cash interest expense on liability related to royalty monetization, $3.9 million of stock-based compensation expense and $3.5 million in unrealized foreign exchange loss. Cash used in operating activities was $6.5 million during the six months ended June 30, 2018. The cash used in operating activities was primarily a result of our $16.2 million net loss from operations and net decrease in assets and liabilities of $1.3 million. The net decrease in operating assets and liabilities during the six months ended June 30, 2018 was primarily driven by decreases in accrued expenses and other assets offset by increases to accounts payable and prepaid expenses and other current assets. This decrease was offset by non-cash items, including $6.7 million in unrealized foreign exchange loss, $2.2 million of stock-based compensation expense and $2.0 million of non cash interest expense on liability related to royalty monetization.

Investing activities

Cash used in investing activities of $0.4 million during the six months ended June 30, 2019 was primarily due to the purchase of property, plant and equipment. Cash used in investing activities of $0.1 million during the six months ended June 30, 2018 was primarily due to the purchase of property, plant and equipment.

Financing activities

Cash provided by financing activities of $22.2 million during the six months ended June 30, 2019 was primarily related to proceeds from the issuance of common stock, net of issuance costs of $20.8 million and proceeds from exercise of options of $1.4 million. Cash provided by financing activities of $138.9 million during the six months ended June 30, 2018 was primarily related to proceeds from the issuance of common stock, net of issuance costs of $94.1 million and royalty monetization of $44.5 million.

Funding Requirements

Cash used to fund operating expenses is affected by the timing of when we pay expenses, as reflected in the change in our outstanding accounts payable and accrued expenses. We believe that our existing cash and cash equivalents will be sufficient to meet our projected operating requirements at least into 2021, including for our Phase 3 clinical program for odevixibat in PFIC, but we will need additional financing to develop odevixibat for the treatment of one or more pediatric liver diseases in 2020. However, our operating plans may change as a result of many factors, including those described below, and we may need additional funds sooner than planned to meet operational needs and capital requirements. In addition, if the conditions for raising capital are favorable we may seek to raise additional funds at any time.

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Our future funding requirements will depend on many factors, including the following:

- the costs, design, duration and any potential delays of the Phase 3 clinical trial of odevixibat;
- the scope, number, progress, duration, cost, results and timing of clinical trials and nonclinical studies of our current or future product candidates;
- whether and to what extent milestone events are achieved under our license agreement with EA Pharma, our RIAA with HCR or any potential future licensee or collaborator;
- the outcomes and timing of regulatory reviews, approvals or other actions;

- our ability to obtain marketing approval for our product candidates;
- our ability to establish and maintain additional licensing, collaboration or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- the success of any other business, product or technology that we acquire or in which we invest;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio;
- our ability to manufacture any approved products on commercially reasonable terms;
- our ability to establish a sales and marketing organization or suitable third-party alternatives for any approved product;
- the number and characteristics of product candidates and programs that we pursue;
- the costs of acquiring, licensing or investing in businesses, product candidates and technologies;
- our need and ability to hire additional management and scientific and medical personnel;
- the costs to operate as a public company in the United States, including the need to implement additional financial and reporting systems and other internal systems and infrastructure for our business;
- market acceptance of our product candidates, to the extent any are approved for commercial sale; and
- the effect of competing technological and market developments.

We cannot determine precisely the completion dates and related costs of our development programs due to inherent uncertainties in outcomes of clinical trials and the regulatory approval process. We cannot be certain that we will be able to successfully complete our research and development programs or establish licensing, collaboration or similar arrangements for our product candidates. Our failure or the failure of any current or potential future licensee to complete research and development programs for our product candidates could have a material adverse effect on our financial position or results of operations.

We expect to continue to incur losses. Our ability to achieve and maintain profitability is dependent upon the successful development, regulatory approval and commercialization of our product candidates and achieving a level of revenues adequate to support our cost structure. We may never achieve profitability.

If the conditions for raising capital are favorable, we may seek to finance future cash needs through public or private equity or debt offerings or other financings. Additionally, if we need to raise additional capital to fund our operations, complete clinical trials, or potentially commercialize our product candidates, we may likewise seek to finance future cash needs through public or private equity or debt offerings or other financings. The necessary funding may not be available to us on acceptable terms or at all.

We filed a new universal shelf registration on Form S-3 with the SEC on March 6, 2019, which was declared effective on April 30, 2019, pursuant to which we registered for sale up to $200 million of any combination of our common stock, preferred stock, debt securities, warrants, rights and/or units from time to time and at prices and on terms that we may determine, which we refer to as the 2019 Form S-3. On March 6, 2019, we terminated the 2017 Sales Agreement and entered into a new sales agreement, which we refer to as the 2019 Sales Agreement, with respect to an at-the-market offering program under which we may offer and sell, from time to time at our sole discretion, shares of our common stock having an aggregate value up to $50.0 million. In May 2019, we sold 637,367 shares of our common stock under the 2019 Sales Agreement for an aggregate of $21.4 million of gross proceeds, which results in $178.6 million of securities remaining available for issuance under the 2019 Form S-3, including $28.6 million of shares of

common stock remaining available for issuance under the 2019 Sales Agreement. We make no assurances as to the continued effectiveness of the 2019 Form S-3. No additional securities registered under the 2017 Form S-3 will be offered or sold.

The sale of additional equity or convertible debt securities may result in significant dilution to our stockholders, and the terms may include liquidation or other preferences that adversely affect the rights of our stockholders. The incurrence of additional debt financing would result in debt service obligations and the instruments governing such debt may provide for operating and financing covenants that would restrict our operations. We may also seek to finance future cash needs through potential future licensing, collaboration or similar arrangements. These arrangements may not be available on acceptable terms or at all, and we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If adequate funds are not available, we may be required to delay, reduce the scope of or eliminate our development programs or obtain funds through third-party arrangements that may require us to relinquish rights to certain product candidates that we might otherwise seek to develop or commercialize independently.

Off-Balance Sheet Arrangements

We have no off-balance sheet arrangements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Not required for smaller reporting companies.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our principal executive officer and principal financial officer, after evaluating the effectiveness of our disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) as of the end of the period covered by this Form 10-Q, have concluded that, based on such evaluation and as a result of the material weaknesses discussed in our "Management's Report on Internal Control over Financial Reporting" in our Form 10-K for the year ended December 31, 2018 and below, our disclosure controls and procedures were not effective to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms, and is accumulated and communicated to our management, including our principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure.

Material Weaknesses and Remediation of Material Weaknesses

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act.

Our management assessed the effectiveness of the Company's internal control over financial reporting as of June 30, 2019. In making this assessment, our management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control-Integrated Framework (2013 framework).

Based on our assessment, our management concluded that the material weaknesses reported in our Annual Report on Form 10-K for the year ended December 31, 2018 remain un-remediated as of June 30, 2019.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. As previously disclosed, the identified material weaknesses relate to our internal control processes and involve the control environment, risk assessment, control activity and monitoring activities. During the six-month period ended June 30, 2019, significant work has been undertaken to remediate the causes of the material weaknesses. Specifically, we have increased the staff of our finance organization including hiring

individuals with experience in U.S. GAAP and SEC reporting and/or skills in and ability to focus on internal control over financial reporting matters. Revised processes and redesigned financial reporting controls have been implemented. General information technology controls to support the effective operation of financial controls have been enhanced to address insufficient design. The material weaknesses will not be considered remediated until the redesigned and enhanced controls operate for a sufficient period of time and management has concluded, through testing, that the controls are operating effectively. Because of the material weaknesses described above, our management believe that, as of June 30, 2019, our internal control over financial reporting was not effective.

Our management remains committed to remediating to ensure that we become compliant with the requirements of Section 404 of the Sarbanes-Oxley Act 2002. Even though significant progress has been made to strengthen our controls, further remediation may be needed. As we continue to evaluate and work to improve our internal control over financial reporting, our management may take additional measures.

Changes in Internal Control over Financial Reporting

Other than as described above, there were no changes in our internal control over financial reporting identified in connection with the evaluation of such internal control that occurred during the three months ended June 30, 2019 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

On February 19, 2019, the Company filed a complaint for breach of contract and breach of implied covenant of good faith and fair dealing against Ferring International Center S.A. (the "Respondent") in the United States District Court for the Southern District of New York. Based on procedural considerations, we decided to refile the complaint in the Supreme Court of the State of New York, County of New York on April 26, 2019. We previously entered into the License Agreement, dated July 2, 2012, as amended as of October 2013 (the "License Agreement"), by and between Respondent and us, pursuant to which Respondent, among other things, conducted two Phase 3 clinical trials to evaluate the efficacy and safety of elobixibat as a treatment for chronic idiopathic constipation, known as Echo 1 and Echo 2, which ended in 2014. As previously disclosed, Respondent stopped Echo 1 and Echo 2 early citing an issue related to the distribution of study drug to study sites that was unrelated to the performance of elobixibat and terminated the License Agreement. The complaint alleges that Respondent breached its obligations under the License Agreement to (1) make earned milestone payments, (2) use good clinical practices, good laboratory practices and good manufacturing practices, and (3) use commercially reasonable efforts. The complaint also alleges that Respondent violated the covenant of good faith and fair dealing implied in the License Agreement. In the complaint, the Company is seeking, among other things, compensatory damages of at least € 37 million (Converted to $42.2 million as of June 30, 2019). On July 31, 2019 Respondent filed a motion to dismiss the complaint.

The Company has retained outside counsel under a contingency fee arrangement, and as a result, the Company will not incur attorneys' fees for litigating the matter, but counsel will receive a contingent fee of 33 1/3% of the net recovery (after deduction of expenses) in the event a recovery is received.

Due to their nature, it is difficult to predict the outcome, or the costs involved in any litigation. Furthermore, Respondent may have significant resources and interest to litigate and therefore, although we have a contingency fee arrangement, this litigation could be protracted and may ultimately involve significant legal expenses.

Item 1A. Risk Factors

Except as set forth below, there have been no material changes to the risk factors described in our Annual Report on Form 10-K for the year ended December 31, 2018, filed with the Securities and Exchange Commission on March 6, 2019.

We face substantial competition, which may result in others discovering, developing or commercializing products to treat our target indications or markets before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide.

Competitors may also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining approvals from regulatory authorities and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies that may be complementary to or necessary for our programs.

Our commercial opportunities could be reduced or eliminated if our competitors develop and commercialize products that are more effective, safer, have fewer or less severe side effects, are approved for broader indications or patient populations, or are more convenient or less expensive than any products that we develop and commercialize. Our competitors may also obtain marketing approval for their products more rapidly than we may obtain approval for our products, which could result in our competitors establishing a strong market position before we are able to enter the market.

In particular, we are aware of other companies that are developing product candidates that, like our product candidates odevixibat and elobixibat, act via IBAT inhibition. SHP625, also known as maralixibat, and formerly known as LUM001 from Lumena Pharmaceuticals, was studied in Phase 2 clinical trials in PFIC and ALGS. In June 2016, Shire announced that the FDA granted breakthrough therapy designation for SHP625 for PFIC, type 2. Shire's SHP626, also known as volixibat, was in Phase 2 development as a treatment for NASH. In November 2018, Shire announced that it licensed exclusive global rights to maralixibat and volixibat to Mirum Pharmaceuticals, Inc. Mirum has announced that it commenced enrollment in a Phase 3 clinical trial of maralixibat in PFIC in the second quarter of 2019 and plans to initiate a Phase 3 clinical trial in ALGS in the first half of 2020. GlaxoSmithKline's GSK2330672, which GlaxoSmithKline has announced an intent to divest, is at the Phase 2 clinical development stage as a treatment for pruritus in patients with PBC. Given the small size of the patient populations for the indications we are targeting, any increase in the number of competitors who are attempting to recruit patients for trials in the same or similar indications as we are may have a material adverse effect on our business. See also "***If we experience delays or difficulties in the enrollment of patients in our Phase 3 clinical trial of A4250 in patients with PFIC, our receipt of marketing approval for A4250 could be delayed or prevented.***"

Item 6. Exhibits

Exhibit No.	Description	Filed Herewith	Incorporated by Reference Herein from Form or Schedule	Filing Date	SEC File/ Req. Number
10.1*	Albireo Pharma, Inc. 2018 Equity Incentive Plan, as amended		8-K (Exhibit 10.1)	6/18/2019	001-33451
31.1	Certification of the Registrant's Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.	X			
31.2	Certification of the Registrant's Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.	X			
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	X			
101	The following materials from the Registrant's Quarterly Report on Form 10-Q for the quarter ended June 30, 2019, formatted in XBRL (eXtensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets (unaudited) at June 30, 2019 and December 31, 2018, (ii) Condensed Consolidated Statements of Operations (unaudited) for the three and six months ended June 30, 2019 and 2018, (iii) Condensed Consolidated Statements of Comprehensive Income (Loss) (unaudited) for the three and six months ended June 30, 2019 and 2018, (iv) Condensed Consolidated Statement of Stockholders' Equity (unaudited) for the three and six months ended June 30, 2019 and 2018, (v) Condensed Consolidated Statements of Cash Flows (unaudited) for the three and six months ended June 30, 2019 and 2018, and (vi) Notes to Condensed Consolidated Financial Statements (unaudited).	X			

* Management contract or compensatory plan or arrangement

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.

ALBIREO PHARMA, INC.

Dated: August 8, 2019

By: /s/ Ronald H.W. Cooper
Ronald H.W. Cooper
President and Chief Executive Officer

Exhibit 31.1

CERTIFICATIONS UNDER SECTION 302

I, Ronald H.W. Cooper, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Albireo Pharma, Inc.;

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

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

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

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

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

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

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

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent 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: August 8, 2019

/s/ Ronald H.W. Cooper
Ronald H.W. Cooper
President and Chief Executive Officer
(principal executive officer)

Exhibit 31.2

CERTIFICATIONS UNDER SECTION 302

I, Simon N.R. Harford, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Albireo Pharma, Inc.;

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

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

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

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

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

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

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

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent 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: August 8, 2019

/s/ Simon Harford
Simon N.R. Harford
Chief Financial Officer and Treasurer (principal financial officer and principal accounting officer)

Exhibit 32.1

CERTIFICATIONS UNDER SECTION 906

Pursuant to section 906 of the Sarbanes-Oxley Act of 2002 (subsections (a) and (b) of section 1350, chapter 63 of title 18, United States Code), each of the undersigned officers of Albireo Pharma, Inc., a Delaware corporation (the "Company"), does hereby certify, to such officer's knowledge, that:

The Quarterly Report for the quarter ended June 30, 2019 (the "Form 10-Q") of the Company fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, and the information contained in the Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: August 8, 2019

/s/ Ronald H.W. Cooper
Ronald H.W. Cooper
President and Chief Executive Officer
(principal executive officer)

Dated: August 8, 2019

/s/ Simon Harford
Simon N.R. Harford
Chief Financial Officer and Treasurer
(principal financial officer and principal accounting officer)