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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 March 31, 2021

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 1000, 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 April 29, 2021, there were 19,194,729 shares of Common Stock, $0.01 par value per share, outstanding.

Albireo Pharma, Inc.

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 operating 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 Bylvay™ (odevixibat), elobixibat, A3907, A2342 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 BOLD, our pivotal clinical trial of Bylvay in patients with biliary atresia or ASSERT, our pivotal trial of Bylvay in Alagille syndrome, or ALGS) for submission, review 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;

- access to the Expanded Access Program (EAP) for Bylvay, or the potential commercial launch of Bylvay in patients with PFIC, subject to regulatory approval;

- any payment that 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;

- the potential impacts of the COVID-19 pandemic on our business operations or financial condition;

- 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 BOLD, our pivotal trial of Bylvay in biliary atresia, and ASSERT our pivotal trial of Bylvay in ALGS, or any other trials that will be required to obtain

marketing approval for Bylvay to treat patients with PFIC, biliary atresia, ALGS or any other pediatric cholestatic liver disease or for A3907 and A2342 as potential treatments for adult liver and viral diseases;

- whether favorable findings from clinical trials of Bylvay to date, including findings in our completed Phase 3 clinical trial in PFIC and findings in indications other than PFIC, will be predictive of results from future clinical trials, including our pivotal trial of Bylvay in biliary atresia and pivotal trial of Bylvay in ALGS;
- 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 our completed Phase 3 trial in patients with PFIC are sufficient, even though such primary endpoint was met with statistical significance, to support approval of Bylvay in the United States, or U.S., or the European Union, or E.U., to treat PFIC, a symptom of PFIC, a specific PFIC subtype(s) or otherwise; and whether either agency will complete their respective reviews within the target timelines, including the FDA's Prescription Drug User Fee Act goal date of July 20, 2021, as a potential result of the impact of the COVID-19 pandemic 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, our pivotal trial of Bylvay in biliary atresia and our pivotal trial of Bylvay in ALGS, and the outcomes of such trials;
- delays or other challenges in the recruitment of patients for the pivotal trial of Bylvay in biliary atresia and the pivotal trial of Bylvay in ALGS;
- whether Bylvay 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 Bylvay, if any, will be valuable to us, and, if necessary, whether the rare pediatric disease priority review voucher program will be renewed beyond 2026;
- the COVID-19 pandemic, which may negatively impact the conduct of, and the timing of initiation, enrollment, completion and reporting with respect to, our clinical trials; negatively impact the supply of drug product for our clinical and preclinical programs; and/or result in other adverse impacts on our business;
- 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 Bylvay, A3907, A2342 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 Bylvay, A3907, A2342 or any of our other product candidates;
- the extent to which our agreement with EA Pharma for elobixibat generates 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;
- whether we are able to effectively commercialize Bylvay in patients with PFIC, if approved by the FDA or EMA;

- 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;
- 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;
- whether we are able to maintain compliance with the terms and conditions of our loan and security agreement with Hercules Capital, Inc.;
- our ability to expand and protect our intellectual property estate;
- regulatory developments in the United States and other countries;
- the effectiveness of our internal control over financial reporting;
- 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, 2020, 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 data)

(unaudited)

	March 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 217,081	$ 251,272
Prepaid expenses and other current assets	8,884	10,593
Total current assets	225,965	261,865
Property and equipment, net	440	478
Goodwill	17,260	17,260
Other assets	6,154	6,004
Total assets	$ 249,819	$ 285,607
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 6,577	$ 5,283
Accrued expenses	13,628	19,051
Current portion of note payable, net of discount	600	—
Other current liabilities	1,406	948
Total current liabilities	22,211	25,282
Liability related to sale of future royalties	67,113	65,894
Note payable, net of discount	9,136	9,621
Other long-term liabilities	3,441	3,579
Total liabilities	101,901	104,376
Stockholders' Equity:		
Preferred stock, $0.01 par value per share — 50,000,000 authorized at March 31, 2021 and December 31, 2020; 0 and 0 issued and outstanding at March 31, 2021 and December 31, 2020, respectively	—	—
Common stock, $0.01 par value per share — 30,000,000 authorized at March 31, 2021 and December 31, 2020; 19,192,805 and 19,107,040 issued and outstanding at March 31, 2021 and December 31, 2020, respectively	192	191
Additional paid-in capital	459,937	456,472
Accumulated other comprehensive loss	(1,658)	(8,612)
Accumulated deficit	(310,553)	(266,820)
Total stockholders' equity	147,918	181,231
Total liabilities and stockholders' equity	$ 249,819	$ 285,607

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 March 31,	
	2021	2020
Revenue	$ 1,966	$ 1,549
Operating expenses:		
Research and development	19,943	16,130
General and administrative	15,273	8,153
Other operating expense, net	6,528	6,816
Total operating expenses	41,744	31,099
Operating loss	(39,778)	(29,550)
Interest expense, net	(3,955)	(1,938)
Net loss	$ (43,733)	$ (31,488)
Net loss per common share - basic and diluted	$ (2.29)	$ (2.23)
Weighted-average common shares used to compute basic and diluted net loss per common share	19,131,557	14,132,217

See accompanying notes to Condensed Consolidated Financial Statements.

Albireo Pharma, Inc.

Condensed Consolidated Statements of Comprehensive Loss

(in thousands)

(unaudited)

	Three Months Ended March 31,	
	2021	2020
Net loss	$ (43,733)	$ (31,488)
Other comprehensive (loss) income:		
Foreign currency translation adjustment	6,954	6,287
Total other comprehensive income	6,954	6,287
Total comprehensive loss	$ (36,779)	$ (25,201)

See accompanying notes to Condensed Consolidated Financial Statements.

Albireo Pharma, Inc.

Condensed Consolidated Statements of Stockholders' Equity

(in thousands, except share data)

(unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance--December 31, 2020	19,107,040	$ 191	$ 456,472	$ (8,612)	$ (266,820)	$ 181,231
Stock-based compensation expense	—	—	3,062	—	—	3,062
Exercise of options and vesting of RSUs	85,765	1	403	—	—	404
Other comprehensive income	—	—	—	6,954	—	6,954
Net loss	—	—	—	—	(43,733)	(43,733)
Balance--March 31, 2021	19,192,805	$ 192	$ 459,937	$ (1,658)	$ (310,553)	$ 147,918

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance--December 31, 2019	12,749,443	$ 127	$ 245,769	$ 6,452	$ (159,187)	$ 93,161
Stock-based compensation expense	—	—	2,381	—	—	2,381
Exercise of options and vesting of RSUs	37,662	—	94	—	—	94
Issuance of common stock, net of costs	2,190,750	22	42,977	—	—	42,999
Other comprehensive income	—	—	—	6,287	—	6,287
Net loss	—	—	—	—	(31,488)	(31,488)
Balance--March 31, 2020	14,977,855	$ 149	$ 291,221	$ 12,739	$ (190,675)	$ 113,434

See accompanying notes to Condensed Consolidated Financial Statements.

Albireo Pharma, Inc.

Condensed Consolidated Statements of Cash Flows

(in thousands)

(unaudited)

	Three Months Ended March 31,	
	2021	2020
Cash flows from operating activities:		
Net loss	$ (43,733)	$ (31,488)
Adjustments to reconcile net loss to net cash used in operating activities:		
Accretion of liability related to sale of future royalties	3,100	2,040
Accretion of debt discount and amortization of issuance costs	115	—
Depreciation and amortization	37	39
Share based compensation expense	3,062	2,381
Foreign currency adjustments	6,479	6,544
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	1,669	1,283
Other assets	152	135
Accounts payable	1,362	2,233
Accrued expenses	(5,245)	(6,987)
Other current and long-term liabilities	(1,714)	24
Net cash used in operating activities	(34,716)	(23,796)
Cash flows from financing activities:		
Proceeds from issuance of common stock, net of issuance costs	—	42,999
Proceeds from exercise of options	404	94
Net cash provided by financing activities	404	43,093
Effect of exchange rate changes on cash and cash equivalents	121	(625)
Net (decrease) increase in cash and cash equivalents	(34,191)	18,672
Cash and cash equivalents—beginning of period	251,272	131,843
Cash and cash equivalents—end of period	$ 217,081	$ 150,515

See accompanying notes to Condensed Consolidated Financial Statements.

Albireo Pharma, Inc.

Notes to Condensed Consolidated Financial Statements

(unaudited)

1. Summary of significant accounting policies and basis of presentation

Organization

Albireo Pharma, Inc. (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 product candidate, a Phase 1 product candidate and elobixibat, which is approved in Japan for the treatment of chronic constipation. Bylvay, the Company's Phase 3 lead product candidate, is in development for the treatment of patients with progressive familial intrahepatic cholestasis (PFIC), biliary atresia and Alagille syndrome (ALGS), each a rare, life-threatening disorder affecting young children.

Since its inception, the Company has devoted substantially all of its resources to its research and development efforts, including activities to develop its product candidates and to prepare for potential commercialization of Bylvay in PFIC in 2021. and to provide general and administrative support for these operations.

The Company has primarily funded its operations with proceeds from the sales of common stock, the sale of royalties to HealthCare Royalty Partners III, L.P. (HCR) and proceeds from the issuance of debt. As of March 31, 2021, the Company has raised an aggregate of $356.7 million through the issuance common stock, net of issuance costs, $59.3 million from the sale of its future royalties and $9.5 million through the Loan and Security Agreement, net of issuance costs.

The Company has incurred significant operating losses and negative cash flows from operations since inception. The Company expects to continue to incur significant expenses and operating losses for the foreseeable future. In addition, the Company anticipates that its expenses will increase significantly in connection with ongoing activities to support the launch of Bylvay for PFIC and the advancement of Bylvay into later stage clinical trials and providing administrative support.

The Company does not expect to generate significant revenue from sales of Bylvay in 2021. As a result, the Company will need substantial additional funding to support its continued operations and growth strategy. Until such a time as the Company can generate significant revenue from product sales, if ever, the Company expects to finance its operations through the sale of equity, debt financings or other capital sources, including collaborations with other companies or other strategic transactions. The Company may be unable to raise additional funds or enter into such other agreements on favorable terms, or at all. If the Company fails to raise capital or enter into such agreements as, and when, needed, the Company may have to significantly delay, scale back or discontinue the development and commercialization of one or more of its product candidates or delay its pursuit of potential in-licenses or acquisitions.

As of March 31, 2021, the Company had cash and cash equivalents of $217.1 million. Management believes that its cash and cash equivalent resources at March 31, 2021, will be sufficient to allow the Company to fund its current operating plan through at least the next twelve months from the issuance of these financial statements.

The Company is subject to those risks associated with any biopharmaceutical company that has substantial expenditures for research and development. There can be no assurance that the Company's research and development projects will be successful, that products developed will obtain necessary regulatory approval, or that any approved product will be commercially viable. In addition, the Company operates in an environment of rapid technological

change and is largely dependent on the services of its employees and consultants. If the Company fails to become profitable or is unable to sustain profitability on a continuing basis, then it may be unable to continue its operations at planned levels and be forced to reduce its operations.

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, 2020. In the opinion of management, all adjustments (including normal recurring adjustments) considered necessary for fair presentation have been included in the Condensed Consolidated Financial Statements. The results of operations for the three months ended March 31, 2021 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.

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 Condensed Consolidated Financial Statements include the accounts of the Company 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 (income) 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 March 31, 2021 and December 31, 2020;

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 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, including its clinical trial accruals, realizability of 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

For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

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 has completed all of its performance obligations under the Agreement.

As of March 31, 2021, the Company is eligible to receive an additional regulatory-based milestone payment under the Agreement of $5.0 million 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.

The Agreement will continue until the last royalty period for any product in the territory, which is defined as the period when there are no remaining patent rights or regulatory exclusivity in place for any products subject to royalties. EA Pharma may terminate the Agreement upon 180 days’ prior written notice to the Company. Either party may terminate the Agreement for the other party’s uncured material breach or insolvency and in certain other circumstances agreed to by the parties.

Monetization of Future Royalties

In December 2017, the Company entered into a royalty interest acquisition agreement (RIAA) with 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. In January 2018, the Company received $44.5 million from HCR, net of certain transaction expenses, under the RIAA. On June 8, 2020, the parties entered into an amendment to the RIAA pursuant to which HCR agreed to pay the Company an additional $14.8 million, net of certain transaction expenses, in exchange for the elimination of the (i) $78.8 million cap amount on HCR's rights to receive royalties on sales in Japan and sales milestones for elobixibat in certain other territories that may become payable by EA Pharma and (ii) the $15.0 million payable to the Company if a specified sales milestone is achieved for elobixibat in Japan. 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 and milestones as revenue. Upon receipt of the payments from HCR the Company recorded net cash totaling $59.3 million as a liability related to sale of future royalties (royalty obligation). The royalty obligation will be amortized using the effective interest rate method.

The following table shows the activity within the liability account for the three month period ended March 31, 2021:

	March 31, 2021 (in thousands)
Liability related to sale of future royalties—beginning balance	$ 68,594
Accretion of interest expense on liability related to royalty monetization	3,100
Repayment of the liability	(2,665)
Liability related to sale of future royalties—ending balance	$ 69,029
Less current portion classified within accrued expenses	(1,916)
Net ending liability related to sale of future royalties	$ 67,113

The Company records estimated royalties due for the current period in accrued 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 accretion 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. The sum of these amounts less the $59.3 million proceeds the Company received will be recorded as interest expense over the life of the royalty obligation. At March 31, 2021, the Company's estimate of its total interest expense resulted in an annual effective interest rate of approximately 18.8%.

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.

Recent accounting pronouncements

There are no recently issued accounting pronouncements the Company has not yet adopted that will materially impact the Company's consolidated financial statements.

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.

The following tables represent information about the Company's financial assets that are measured at fair value on a recurring basis (in thousands):

	March 31, 2021		
	Level 1	Level 2	Level 3
Cash Equivalents:			
Money market funds	$ 215,008	$ —	$ —
Total	$ 215,008	$ —	$ —

	December 31, 2020		
	Level 1	Level 2	Level 3
Cash Equivalents:			
Money market funds	$ 242,854	$ —	$ —
Total	$ 242,854	$ —	$ —

The Company's financial instruments consist mainly of cash equivalents, prepaid expenses and other current assets, accounts payable, accrued expenses, other current liabilities, and note payable. The carrying amounts of cash equivalents, prepaid expenses and other current liabilities, accounts payable, accrued expenses, and other current liabilities approximate their estimated fair value due to their short-term maturities. At March 31, 2021, the Loan and Security Agreement with Hercules Capital, Inc., the Company believes the carrying value approximates the fair value of the note payable.

3. Commitments and contingencies

Agreements with CROs and CMOs

As of March 31, 2021, the Company had various agreements with CROs and CMOs for the conduct of specified research and development activities and based on the terms of the respective agreements, the Company is contractually obligated to make future payments of up to $7.6 million upon the completion of contracted work.

4. Net loss per share

Basic net loss per share, is calculated by dividing the net loss attributable to holders of common stock by the weighted average number of shares of common stock outstanding. Diluted net loss per share, is calculated by dividing the net loss attributable to holders of common stock by the weighted average number of shares of common stock outstanding. If the Company were in a net income position, diluted net income per share would be calculated by dividing the net income attributable to holders of common stock 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 net loss per share and Diluted net loss per share (in thousands, except for share and per share data):

	Three Months Ended March 31,	
	2021	2020
Basic and Diluted net loss per share:		
Numerator		
Net loss	$ (43,733)	$ (31,488)
Denominator		
Weighted average number of shares outstanding	19,131,557	14,132,217
Basic and Diluted net loss per share:	$ (2.29)	$ (2.23)

The following outstanding common stock equivalents were excluded from the computation of Diluted net loss per share for the three months ended March 31, 2021 and 2020 because including them would have been anti-dilutive:

	Three Months Ended March 31,	
	2021	2020
Options to purchase common stock, RSUs and warrants	3,141,689	2,293,790

5. Income taxes

The Company did not record a tax provision or benefit for the three months ended March 31, 2021 or 2020. 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. Note Payable

2020 Loan and Security Agreement

On June 8, 2020, the Company entered into a Loan and Security Agreement with the several banks and other financial institutions or entities from time to time parties to the Loan and Security Agreement, as lenders, or collectively referred to as the Lender, and Hercules Capital, Inc., in its capacity as administrative agent and collateral agent for itself and Lender (in such capacity, the Agent or Hercules). The Loan and Security Agreement provides for term loans in an aggregate principal amount of up to $80.0 million to be delivered in multiple tranches, (the Term Loans). The tranches consist of (i) a term loan advance to Company in an aggregate principal amount of up to $15.0 million, of which (A) the Company agreed to borrow an aggregate principal amount of $10.0 million on the date on which all conditions to the funding of the Term Loans by the Lender were met (the Closing Date), but the Company did not request that the Lender make an additional term loan advance to the Company in an aggregate principal amount of $5.0 million prior to December 15, 2020 as permitted under the agreement, (ii) subject to the achievement of certain initial performance milestones, or Performance Milestone I, the Company has the right to request that the Lender make additional term loan advances to the Company in an aggregate principal amount of up to $20.0 million from January 1, 2021 through December 15, 2021 in minimum increments of $10.0 million, and (iii) subject to the Lender’s investment committee’s sole discretion, the Company has the right to request that the Lender make additional term loan advances to the Company in an aggregate principal amount of up to $45.0 million through March

31, 2022 in minimum increments of $5.0 million. As of March 31, 2021, we borrowed an aggregate principal amount of $10.0 million. An aggregate principal amount of up to $20.0 million remains available for future borrowings upon request and an aggregate principal amount of up to $45.0 million remains available for future borrowing subject to approval from lender. The Company is required to pay an end of term fee ("End of Term Charge") equal to 6.95% of the aggregate principal amount of the Term Loans advances upon repayment.

The Term Loans mature on January 1, 2024, which is extendable to June 1, 2024 upon achievement of Performance Milestone I (the "Maturity Date").

The Term Loan bears interest at an annual rate equal to the greater of 9.15% and 9.15% plus the prime rate of interest minus 3.25%. Borrowings under the Loan and Security Agreement are repayable in monthly interest-only payments through January 1, 2022 and extendable to (i) July 1, 2022 upon achievement of Performance Milestone I and (ii) July 1, 2023 upon achievement of certain additional performance milestones. After the interest-only payment period, borrowings under the Loan and Security Agreement are repayable in equal monthly payments of principal and accrued interest until the Maturity Date. At the Company's option, the Company may elect to prepay all, but not less than all, of the outstanding term loan by paying the entire principal balance and all accrued and unpaid interest thereon plus a prepayment charge equal to the following percentage of the principal amount being prepaid: 2.0% of the principal amount outstanding if the prepayment occurs after the first nine months following the Closing Date, but on or prior to 24 months following the Closing Date, and 1.0% of the principal amount outstanding at any time thereafter but prior to the Maturity Date.

In connection with the Loan and Security Agreement, the Company granted Agent a security interest senior to any current and future debts and to any security interest, in all of the Company's right, title, and interest in, to and under all of Company's property and other assets, and certain equity interests and accounts of Albireo AB, subject to limited exceptions including the Company's intellectual property. The Loan and Security Agreement also contains certain events of default, representations, warranties and non-financial covenants of the Company.

The debt discount and issuance costs are being accreted to the principal amount of debt and being amortized from the date of issuance through the Maturity Date to interest expense using the effective-interest rate method. The effective interest rate of the outstanding debt under the Loan and Security Agreement is approximately 15.3%.

As of March 31, 2021 the carrying value of the note payable consists of the following:

	March 31, 2021 (in thousands)
Note payable, including End of Term Charge	10,695
Debt discount, net of accretion	(959)
Less: current portion, net of discount	(600)
Note payable net of discount, long-term	$ 9,136

During the three months ended March 31, 2021, the Company recognized $0.3 million, of interest expense related to the Loan and Security Agreement. No interest expense was associated with the Loan and Security Agreement for the three months ended March 31, 2020.

Estimated future principal payments due under the Loan and Security Agreement, including the contractual End of Term Charge, are as follows as of March 31, 2021:

	Note Principal Payments (in thousands)
Remainder of 2021	$ —
2022	4,553
2023	4,994
2024	1,148

As of March 31, 2021, based on Level 3 inputs and the borrowing rates available to the Company for loans with similar terms and consideration of the Company's credit risk, the carrying value of the Company's variable interest rate debt, excluding unamortized debt issuance costs, approximated fair value.

Warrants

Under the Loan and Security Agreement, the Company agreed to issue to Hercules warrants (the "Warrants") to purchase a number of shares of common stock equal to 1% of the aggregate amount of the Term Loans that are funded, as such amounts are funded. On the Closing Date, the Company issued a Warrant for 5,311 shares of common stock. The Warrants are exercisable for a period of seven years from the date of the issuance of each Warrant at a per-share exercise price equal to $18.83, subject to certain adjustments as specified in the Warrants. In addition, the Company has granted to the holders of the Warrants certain registration rights. Specifically, the Company has agreed to use its commercially reasonable efforts to (i) file registration statements with the U.S. Securities and Exchange Commission within 60 days following the date of the issuance of each Warrant for purposes of registering the shares of common stock issuable upon exercise of the Warrants for resale by Hercules, and (ii) cause the registration statement to be declared effective as soon as practicable after filing, and in any event no later than 180 days after the date of the issuance of each Warrant.

The Company accounted for the Warrants as equity instruments since they were indexed to the Company's common stock and met the criteria for classification in stockholders' equity. The relative fair value of the Warrants related to the first tranche funding was approximately $0.1 million, and was treated as a discount to the Term Loans. This amount is being amortized to interest expense using the effective interest method over the life of the Term Loans. The Company estimated the fair value of the Warrants using the Black-Scholes option-pricing model.

7. Equity Financings

2020 Underwritten Public Offerings

In February 2020, the Company completed an underwritten public offering of 2,190,750 shares of its common stock under a universal shelf registration statement for net proceeds of approximately $43.0 million, after deducting underwriting discounts and commissions and offering expenses.

8. Stock-based Compensation

For the three months ended March 31, 2021, the Company granted 663,650 options at a weighted average exercise price of $35.49. For the three months ended March 31, 2021, the Company granted 151,100 RSUs.

The Company recorded the following stock-based compensation expense:

	Three Months Ended March 31,	
	2021	2020
Employee awards:		
Research and development expense	$ 1,136	$ 880
General and administrative expense	1,926	1,501
Total stock-based compensation expense	$ 3,062	$ 2,381

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, 2020, 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, 2020, 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. We are pursuing the development of our lead product candidate, Bylvay, for patients with progressive familial intrahepatic cholestasis, or PFIC, a rare, life-threatening genetic disorder affecting young children for which there is currently no approved drug treatment. In September 2020, we announced topline results from our Phase 3 trial in PFIC, and in December 2020, we announced that we submitted a new drug application, or NDA, to the U.S. Food and Drug Administration, or FDA, and a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, seeking approval of Bylvay for the treatment of patients with PFIC, in anticipation of potential regulatory approval, issuance of a rare pediatric disease priority review voucher and commercial launch in the second half of 2021. In January 2021 we announced the FDA accepted our NDA for Bylvay for the treatment of patients with PFIC, which was submitted in November 2020, with priority review and a user fee goal date under the Prescription Drug User Fee Act (PDUFA) of July 20, 2021. There are currently no plans for a FDA advisory committee meeting regarding our NDA. We are also pursuing the development of Bylvay in biliary atresia and in Alagille syndrome, or ALGS, each of which is a rare, life threatening disease that affects the liver and for which there is no approved pharmacologic treatment option. We initiated a pivotal clinical trial of Bylvay in biliary atresia, the BOLD trial, in the first half of 2020, and continue to enroll patients in the trial. We expect topline results from the BOLD trial in 2024. We also initiated a pivotal trial of Bylvay in ALGS, the ASSERT trial, in the fourth quarter of 2020 and have dosed the first patients in the trial. We expect topline results from the ASSERT trial in 2022. Our most advanced product candidate in addition to Bylvay is elobixibat, which is approved in Japan for the treatment of chronic constipation. In August 2020, we announced topline results from our Phase 2 clinical trial as a treatment for nonalcoholic fatty liver disease, or NAFLD, and nonalcoholic steatohepatitis, or NASH, and based on the results of the trial, we decided not to pursue further development of elobixibat in NAFLD or NASH. We are expanding development to compounds that are intended for adult liver and viral diseases. Our lead candidate for adult liver diseases, A3907, is a selective inhibitor of the apical sodium-dependent bile acid transporter (ASBT) that has, based on animal studies, high predicted systemic bioavailability in man. As a result, A3907 has the potential to not only affect the bile acid pool by increased bile acid excretion in the stools but also through other pathways, including increased urinary bile acid excretion. This unique approach may yield greater dosing flexibility, greater efficacy and lower rates of adverse events associated with the category, such as diarrhea. We initiated a Phase 1 clinical trial, with the first patient dosed in the first quarter of 2021, we expect topline results from that trial in 2021. If the Phase 1 trial is successful, we expect to initiate a Phase 2 trial for A3907 in adult liver disease in 2022. A US composition of matter and method of use patent for A3907 was issued with an expiration date between 2039 and 2040, not including patent term extension. We also have a preclinical program in adult liver and viral diseases. Our lead preclinical candidate for adult viral and liver diseases is A2342, a potent small molecule inhibitor of the sodium-taurocholate co-transporting peptide (NTCP). NTCP is a key transporter of bile acids into the liver cells but also the entry mechanism for the hepatitis B (HBV) and hepatitis D (HDV) viruses. A2342 protects primary human hepatocytes from HBV infection in vitro. In addition, A2342 reduces markers of infection in HBV-infected humanized mice. A2342 has demonstrated target engagement in non-human primates with

biomarker increases comparable to increases achieved in humans by a now commercial subcutaneous peptide NTCP inhibitor. We plan to complete IND enabling studies with A2342 this year. Preclinical efforts with other bile acid modulator approaches continue.

Bylvay — Our Lead Product Candidate for PFIC.

In September 2020, we announced topline results from PEDFIC 1, our Phase 3 clinical trial for Bylvay, given once per day as an oral capsule or sprinkled over food, in children ages 6 months to 18 years with PFIC types 1 and 2, which was conducted at 45 global sites. PEDFIC 1 tested two doses of Bylvay, 40 µg/kg/day and 120 µg/kg/day, along with placebo, over a treatment period of 24 weeks. PEDFIC 1, met its two primary endpoints, demonstrating that Bylvay reduced serum bile acid responses, or sBAs, (p=0.003) and improved pruritus assessments (p=0.004) with a single digit diarrhea rate. In the primary analysis, PEDFIC 1 met the U.S regulatory primary endpoint with the proportion of positive pruritus assessments being 53.5% in the Bylvay arms compared to 28.7% in the placebo arm (p=0.004). As a secondary endpoint, 42.9% of patients in the Bylvay arms had a clinically meaningful improvement in the pruritus score, defined as a drop from baseline of 1.0 point or more on the 0-4 point scale, at week 24 compared to 10.5% in the placebo arm (p=0.018). PEDFIC 1 also met the E.U. regulatory primary endpoint with 33.3% of subjects in the Bylvay arms experiencing either a 70% reduction in sBAs or reaching a level of 70 µmol/L compared to no patients in the placebo arm (p=0.003). As an E.U. regulatory secondary endpoint, mean reduction of bile acids was 114.3 µmol/L in the Bylvay arms compared to an increase of 13.1 µmol/L in the placebo arm (p=0.002). Both doses of Bylvay were statistically significant for each of the U.S. and E.U. primary endpoints. Bylvay was well tolerated, with an overall adverse event incidence similar to placebo. There were no drug-related serious adverse events, or SAEs, reported during the study. Diarrhea/frequent bowel movements were the most common treatment-related gastrointestinal adverse events, which occurred in 9.5% of Bylvay treated patients vs. 5.0% of placebo patients. In December 2020, we announced that we submitted an NDA to the FDA and an MAA to the EMA seeking approval of Bylvay for the treatment of PFIC, which affirms our eligibility to apply for a rare pediatric disease priority review voucher. In January 2021, we announced the FDA accepted our NDA for Bylvay for the treatment of patients with PFIC, with priority review and a PDUFA user fee goal date of July 20, 2021. In June 2018, the FDA granted a rare pediatric disease designation to Bylvay for the treatment of PFIC, and we applied for a rare pediatric disease priority review voucher in connection with the submission of our NDA in November 2020. In September 2018, the FDA granted fast track designation to Bylvay for the treatment of pruritus associated with PFIC. In July 2020, we initiated an Expanded Access Program (EAP) for Bylvay in the United States, Canada, Australia and Europe.

PEDFIC 2, our long term, open label extension study, includes a cohort of patients who completed 24 weeks in PEDFIC 1 or moved into PEDFIC 2 after 12 weeks in PEDFIC 1, as well as an additional cohort of PFIC patients who were not eligible for PEDFIC 1. Patients in PEDFIC 2 receive Bylvay 120 µg/kg once per day over 72 weeks. Primary outcome measures in PEDFIC 2 are change in pruritus as indexed by caregiver reported observed scratching using our proprietary PRUCISION instrument, and change in sBAs, in each case from baseline over 72 weeks. In November 2020, we announced interim results from 69 patients through 24 weeks of treatment in PEDFIC 2 that show that the reductions in sBAs and/or pruritus observed in patients receiving Bylvay in PEDFIC 1 was maintained or increased during continued Bylvay treatment in PEDFIC 2. For patients who were treatment naïve to Bylvay (patients who received placebo in PEDFIC 1 or patients who enrolled directly into PEDFIC 2), reductions in sBAs and pruritus were similar to those observed during Bylvay treatment in PEDFIC 1. Continued treatment with Bylvay in PEDFIC 2 resulted in increased growth rates and catch-up growth in children with PFIC. Four patients with PFIC type 3 enrolled into PEDFIC 2 and had 12 weeks of data available at the time of the interim data cut; reductions in both sBAs and pruritus were also observed in these patients. No deaths or treatment related serious adverse events had been reported in PEDFIC 2 at the time of the interim data cut as of July 15, 2020. Bylvay was generally well tolerated; diarrhea was reported in 10.1% of patients, all mild or moderate in severity.

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 75,000 children born worldwide. Based on the published incidence, published regional populations, and estimated median life expectancies, we estimate the prevalence of PFIC across the spectrum of the

disease to be approximately 15,000 patients worldwide, not including China and India, but we are not able to estimate the prevalence of PFIC with precision. We hold global rights to Bylvay unencumbered. Our current plan is to commercialize Bylvay ourselves in the United States and Europe. We have entered into a Co-Promotion Agreement with Travere Therapeutics, Inc. for the co-promotion of Bylvay in the United States. The initial term of the arrangement is two years from launch of Bylvay, terminable at will by either party after one year following launch. We have also entered into license agreements with third parties to commercialize Bylvay in certain other jurisdictions, subject to regulatory approval in those jurisdictions including, Medison Pharma Ltd. in Israel, Gen İlaç ve Sağlık Ürünleri Sanayi ve Ticaret A.Ş. in Turkey and Genpharm Services for Saudi Arabia, Bahrain, Kuwait, Oman, Qatar, and the UAE, and we are 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 France only for PFIC type 3, and 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 Bylvay.

We are also pursuing the development of Bylvay in patients with biliary atresia, another rare, life-threatening disease that affects the liver and for which there is no approved pharmacologic treatment option. In December 2018, the European Commission granted orphan designation to Bylvay for the treatment of biliary atresia, and in January 2019, the FDA granted orphan drug designation to Bylvay for the treatment of biliary atresia. We initiated the BOLD clinical trial, a global pivotal trial and the largest prospective intervention trial ever conducted in biliary atresia, in the first half of 2020. The first patients have been enrolled in the trial, and we plan for full site activation in the first half of 2021, subject to any potential impacts of COVID-19 on the ability of sites to complete required activities. We expect topline results from the BOLD trial in 2024. We believe biliary atresia is one of the most common rare pediatric liver diseases, and is the leading cause of liver transplants in children. Our double-blind, placebo controlled pivotal trial in biliary atresia is designed to enroll approximately 200 patients at 70 sites globally. Patients will receive either placebo or high-dose (120µg/kg) Bylvay once daily. The primary endpoint is survival with native liver after two years of treatment.

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 6 and 10 for every 100,000 live births. We estimate the prevalence of biliary atresia to be approximately 18,100 patients across the U.S. and Europe, and approximately 27,000 combined in other jurisdictions worldwide, but we are not able to estimate the prevalence of biliary atresia with precision. 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.

In addition, we initiated a pivotal trial of Bylvay in ALGS, the ASSERT trial, in the fourth quarter of 2020. The trial is expected to enroll approximately 45 patients aged 0 to 17 years of age with a genetically confirmed diagnosis of ALGS across 35 sites in North America, Europe, Middle East and Asia Pacific. We plan for full site activation in the first half of 2021, subject to any potential impacts of COVID-19 on the ability of sites to complete required activities. The first patients have been dosed in the trial and we expect topline data to be available in 2022, before the announcement of the topline results from the BOLD trial. 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 50,000 children born worldwide. We estimate the prevalence of ALGS to be approximately 11,700 patients across the U.S. and Europe, and approximately 13,000 combined in other jurisdictions worldwide, but we are not able to estimate the prevalence of ALGS with precision. 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 August 2012, the European Commission granted orphan designation to Bylvay for the treatment of ALGS. In October 2018, the FDA granted orphan drug designation to Bylvay for the treatment of ALGS.

We continue to evaluate potential clinical development in other indications, including primary sclerosing cholangitis, which 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 9 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.

Since inception, we have incurred significant operating losses. As of March 31, 2021, we had an accumulated deficit of $310.6 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 March 31, 2021, we had approximately $217.1 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.

Other Operating Expense, net

Other operating expense, net consists primarily of foreign currency exchange gains or losses associated with revaluation of intercompany loans.

Interest Expense, net

Interest expense, net consists primarily of non-cash interest expense recorded in connection with the sale of future royalties, related to sales of elobixibat in Japan in addition to interest expense associated with our note payable. In addition, interest expense, net includes interest income associated with our interest-bearing cash and cash equivalents.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our unaudited condensed consolidated financial statements, which have been prepared in accordance with 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 February 25, 2021, the date we filed our Annual Report on Form 10-K for the year ended December 31, 2020. For more information on our critical accounting policies, refer to our Annual Report on Form 10-K for the year ended December 31, 2020.

Results of Operations

Three Months Ended March 31, 2021 and March 31, 2020

Result of Operations

	Three Months Ended March 31,		Change
	2021	2020	$
	(in thousands)		
Revenue	$ 1,966	$ 1,549	$ 417
Operating Expenses			
Research and development	19,943	16,130	3,813
General and administrative	15,273	8,153	7,120
Other operating expense, net	6,528	6,816	(288)
Total operating expenses	41,744	31,099	10,645
Operating loss	(39,778)	(29,550)	(10,228)
Interest expense, net	(3,955)	(1,938)	(2,017)
Net loss	$ (43,733)	$ (31,488)	$ (12,245)

Revenue

	Three Months Ended March 31,		Change
	2021	2020	$
	(in thousands)		
Revenue	$ 1,966	$ 1,549	$ 417

There was $2.0 million in revenue for the three months ended March 31, 2021 compared with $1.5 million for the three months ended March 31, 2020, an increase of $0.4 million. The higher revenue is due to the estimated royalty revenue to be received from EA Pharma for elobixibat for the treatment of chronic constipation.

Research and development expenses

	Three Months Ended March 31,		Change
	2021	2020	$
	(in thousands)		
Research and development expenses	$ 19,943	$ 16,130	$ 3,813

Research and development expenses were $19.9 million for the three months ended March 31, 2021 compared with $16.1 million for the three months ended March 31, 2020, an increase of $3.8 million. The increased research and development expenses for the 2021 period were principally due to personnel expenses as we continue to increase our headcount and program activities. The increase in program activities related to Bylvay for regulatory submissions in PFIC, the additional indications for biliary atresia and Alagille syndrome, and for A3907 were partially offset by elobixibat and preclinical programs.

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 March 31, 2021 and 2020.

	Three Months Ended March 31,		Change
	2021	2020	$
		(in thousands)	
Direct third-party project costs:			
Bylvay	$ 11,737	$ 8,689	$ 3,048
Elobixibat	—	868	(868)
A3384	—	59	(59)
A3907	1,605	—	1,605
Preclinical	813	1,295	(482)
Total	$ 14,155	$ 10,911	$ 3,244
Other project costs[1]:			
Personnel costs	$ 5,660	$ 3,963	$ 1,697
Other costs[2]	128	1,256	(1,128)
Total	$ 5,788	$ 5,219	$ 569
Total research and development costs	$ 19,943	$ 16,130	$ 3,813

(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 March 31,		Change
	2021	2020	$
		(in thousands)	
General and administrative expenses	$ 15,273	$ 8,153	$ 7,120

General and administrative expenses were $15.3 million for the three months ended March 31, 2021 compared with $8.2 million for the three months ended March 31, 2020, an increase of $7.1 million. The increase is attributable to personnel and related expenses as we continue to increase our headcount, and commercialization readiness activity related to PFIC.

Other operating expense, net

	Three Months Ended March 31,		Change
	2021	2020	$
		(in thousands)	
Other operating expense, net	$ 6,528	$ 6,816	$ (288)

Other operating expense, net totaled $6.5 million for the three months ended March 31, 2021 compared with expense of $6.8 million for the three months ended March 31, 2020. The difference primarily relates to changes in foreign currency exchange rates in the two periods.

Interest expense, net

	Three Months Ended March 31,		Change
	2021	2020	$
		(in thousands)	
Interest expense, net	$ (3,955)	$ (1,938)	$ (2,017)

Interest expense, net totaled $4.0 million for the three months ended March 31, 2021 compared with $1.9 million for the three months ended March 31, 2020. The difference was principally attributable to higher non-cash interest expense recorded in connection with the sale of future royalties related to sales of elobixibat in Japan, in addition to interest expense associated with our note payable.

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 the second half of 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, upfront fees paid upon entering into license agreements, payments received upon the achievement of specified milestone events under license agreements, grants and debt borrowings and the HCR royalty monetization transactions. 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 well as pre-commercialization efforts.

As of March 31, 2021, our cash and cash equivalents were approximately $217.1 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. Additionally, this approval triggered a milestone payment to us from EA Pharma of $11.2 million. In June 2020, we entered into an amendment to the RIAA with HCR pursuant to which HCR agreed to pay us an additional $14.8 million, net of certain transaction expenses in exchange for the elimination of the (i) $78.8 million cap amount on HCR's rights to receive royalties on sales in Japan and sales milestones for elobixibat in certain other territories that may become payable by EA Pharma and (ii) $15.0 million payable to us if a specified sales milestone is achieved for elobixibat in Japan. As of March 31, 2021, we have received approximately $59.3 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 $5.0 million under the amended agreement, if a specified regulatory event is achieved for elobixibat.

In addition, in February 2020, we completed an underwritten public offering of 2,190,750 shares of our common stock under our universal shelf registration statement for net proceeds of approximately $43.0 million.

On May 7, 2020, we filed a new universal shelf registration statement on Form S-3, or the 2020 Form S-3, with the SEC, which was declared effective on May 18, 2020, pursuant to which we registered for sale up to $200.0 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. On May 7, 2020, we also entered into a new 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. This agreement terminated on September 9, 2020.

On June 8, 2020, we entered into a Loan and Security Agreement with the several banks and other financial institutions or entities from time to time parties to the Loan and Security Agreement, as lenders, or collectively

referred to as the Lender, and Hercules Capital, Inc., in its capacity as administrative agent and collateral agent for itself and Lender (in such capacity, the Agent or Hercules). The Loan and Security Agreement provides for term loans in an aggregate principal amount of up to $80.0 million to be delivered in multiple tranches, (the Term Loans). The tranches consist of (i) a term loan advance to Company in an aggregate principal amount of up to $15.0 million, of which (A) we agreed to borrow an aggregate principal amount of $10.0 million on the date on which all conditions to the funding of the Term Loans by the Lender were met (the Closing Date), but we did not request that the Lender make an additional term loan advance to us in an aggregate principal amount of $5.0 million prior to December 15, 2020 as permitted under the agreement, (ii) subject to the achievement of certain initial performance milestones, or Performance Milestone I, we have the right to request that the Lender make additional term loan advances to us in an aggregate principal amount of up to $20.0 million from January 1, 2021 through December 15, 2021 in minimum increments of $10.0 million, and (iii) subject to the Lender's investment committee's sole discretion, we have the right to request that the Lender make additional term loan advances to us in an aggregate principal amount of up to $45.0 million through March 31, 2022 in minimum increments of $5.0 million. As of March 31, 2021, we borrowed an aggregate principal amount of $10.0 million. An aggregate principal amount of up to $20.0 million remains available for future borrowing upon request and an aggregate principal amount of up to $45.0 million remains available for future borrowing subject to approval from lender.

On September 14, 2020, we completed an underwritten public offering of 4,000,000 shares of our common stock under this registration statement. We received net proceeds from this offering of approximately $150.4 million, after deducting underwriting discounts and commissions, but before deducting offering expenses. As of March 31, 2021, $40.0 million of securities remain available for issuance under the 2020 Form S-3.

As of March 31, 2021, $40.0 million of securities remain available for issuance under the 2020 Form S-3.

On February 25, 2021, we filed an automatic shelf registration statement on Form S-3 with the SEC, which became effective upon filing, pursuant to which we registered for sale an unlimited amount 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, so long as we continue to satisfy the requirements of a "well-known seasoned issuer" under SEC rules, which we refer to as the 2021 Form S-3. This registration statement will remain in effect for up to three years from the date it became effective. On February 25, 2021, we also entered into a new sales agreement, which we refer to as the 2021 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 $100 million. We make no assurances as to the continued effectiveness of the 2021 Form S-3.

Cash Flows

Three months ended March 31, 2021 and March 31, 2020

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	$ (34,716)	(23,796)
Financing activities	404	43,093
Total	$ (34,312)	$ 19,297
Effect of exchange rate changes on cash and cash equivalents	121	(625)
Net (decrease) increase in cash and cash equivalents	(34,191)	18,672

Operating activities

Cash used in operating activities of $34.7 million during the three months ended March 31, 2021 was primarily a result of our $43.7 million net loss from operations and a net decrease in assets and liabilities of $3.8 million. The net decrease in operating assets and liabilities during the three months ended March 31, 2021 was primarily driven by decreases in accrued expenses, other current and long-term liabilities, prepaid expenses and other current assets, offset by increases in accounts payable. This decrease was offset by non-cash items, including $6.5 million of foreign currency adjustments, $3.1 million of stock-based compensation expense, and $3.1 million of accretion of liability related to sale of future royalties. Cash used in operating activities of $23.8 million during the three months ended March 31, 2020 was primarily a result of our $31.5 million net loss from operations and a net decrease in assets and liabilities of $3.3 million. The net decrease in operating assets and liabilities during the three months ended March 31, 2020 was primarily driven by decreases in accrued expenses and prepaid expenses and other current assets, and an increase to accounts payable. This decrease was offset by non-cash items, including $6.5 million of foreign currency adjustments, $2.4 million of stock- based compensation expense and $2.0 million of non-cash interest on liability related to sale of future royalties.

Financing activities

Cash provided by financing activities of $0.4 million during the three months ended March 31, 2021 was primarily related to proceeds from exercise of options. Cash provided by financing activities of $43.1 million during the three months ended March 31, 2020 was primarily related to proceeds from the issuance of common stock, net of issuance costs of $43.0 million and proceeds from exercise of options of $0.1 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 into 2023 including for the commercial launch of Bylvay, if approved, for our open-label trial in PFIC for Bylvay, in addition to Phase 3 clinical programs for Bylvay in biliary atresia and ALGS, as well as A3907, A2342 and our preclinical programs. We anticipate an operating cash burn rate in the range of $130.0 to $135.0 million in 2021. 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.

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

- any unfavorable development or delay in our Bylvay program in PFIC, including the review or approval of our Bylvay marketing applications in the United States and Europe for PFIC and the costs and timing of our pre-commercialization preparations;
- the costs, design, duration and any potential delays of the pivotal clinical trial of Bylvay in biliary atresia and the pivotal clinical trial of Bylvay in ALGS;
- the scope, number, progress, initiation, 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 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 current and potential impacts of the COVID-19 pandemic on our business;
- 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 dates of potential regulatory approval of Bylvay in PFIC or the dates of our potential commercial launch, or 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 pre-commercialization activities or 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 have an effective universal shelf registration statement on Form S-3 with the SEC, 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,. As of March 31, 2021, $40.0 million of securities remain available for issuance under the shelf registration statement, which we refer to as the 2020 Form S-3. On February 25, 2021, we filed an automatic shelf registration statement on Form S-3 with the SEC, pursuant to which we registered for sale an unlimited amount 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, so long

as we continue to satisfy the requirements of a “well-known seasoned issuer” under SEC rules, which we refer to as the 2021 Form S-3, including up to $100 million of our common stock pursuant to the new sales agreement with respect to an at-the-market offering program. We make no assurances as to the continued effectiveness of the 2021 Form S-3.

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. In addition, following the closing of our September 2020 public offering of common stock, we have a limited number of authorized shares of common stock available for future issuance that are not already issued or reserved for issuance. 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.

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

As of March 31, 2021, 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, our disclosure controls and procedures were 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.

Changes in Internal Control over Financial Reporting

There were no changes in our internal controls over financial reporting identified in connection with the evaluation of such internal controls that occurred during the three months ended March 31, 2021 that have materially affected, or are reasonably likely to materially affect, our internal controls over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

We are not currently a party to any material legal proceedings.

Item 1A. Risk Factors

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, 2020, filed with the Securities and Exchange Commission, or SEC, on February 25, 2021.

Item 6. Exhibits

Exhibit No.	Description	Filed Herewith	Incorporated by Reference Herein from Form or Schedule	Filing Date	SEC File/ Req. Number
10.1*	Nonemployee Director Compensation Policy.	X			
10.2*	Sales Agreement, dated February 25, 2021 by and between the Registrant and Cowen and Company, LLC.		8-K (Exhibit 10.1)	2/25/2021	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 March 31, 2021, formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets (unaudited) at March 31, 2021 and December 31, 2020, (ii) Condensed Consolidated Statements of Operations (unaudited) for the three months ended March 31, 2021 and 2020, (iii) Condensed Consolidated Statements of Comprehensive Loss (unaudited) for the three months ended March 31, 2021 and 2020, (iv) Condensed Consolidated Statement of Stockholders' Equity (unaudited) for the three months ended March 31, 2021 and 2020, (v) Condensed Consolidated Statements of Cash Flows (unaudited) for the three months ended March 31, 2021 and 2020, and (vi) Notes to Condensed Consolidated Financial Statements (unaudited).	X			
104	Cover Page Interactive Date File (formatted as Inline XBRL and contained in Exhibit 101).	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: May 6, 2021

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

ALBIREO PHARMA, INC.

NONEMPLOYEE DIRECTOR COMPENSATION POLICY

(Adopted January 23, 2017, Last modified March 4, 2021)

The Board of Directors of Albireo Pharma, Inc. (the "Company") has approved the following Nonemployee Director Compensation Policy (this "Policy") to provide an inducement to obtain and retain the services of qualified persons to serve as members of the Company's Board of Directors. The Policy establishes compensation to be paid to nonemployee directors of the Company.

Applicable Persons

This Policy shall apply to each director of the Company who is not an employee of, or compensated consultant to, the Company or any Affiliate (each, an "Outside Director"). "Affiliate" shall mean an entity which is a direct or indirect parent or subsidiary of the Company, as determined pursuant to Section 424 of the Internal Revenue Code of 1986, as amended.

Compensation

A. Equity Grants

1. Annual Stock Option Grants

Each Outside Director shall be granted, automatically and without any action on the part of the Board of Directors, under the Company's 2018 Equity Incentive Plan or a successor plan (the "Equity Plan"), a nonqualified stock option to purchase 8,000 shares of the Company's common stock, par value $0.01 per share ("Common Stock"), each year on the fifth (5th) business day after the Company's annual meeting of stockholders (the "Annual Stock Options"); provided, however, that if there has been no annual meeting of stockholders held by the first business day of the third fiscal quarter, each Outside Director shall be granted, automatically and without any action on the part of the Board of Directors, such Annual Stock Option on the first business day of the third fiscal quarter of such year.

2. Initial Stock Option Grants for Newly Appointed or Elected Directors

Each new Outside Director shall be granted, automatically and without any action on the part of the Board of Directors, under the Equity Plan, a nonqualified stock option to purchase 16,000 shares of Common Stock on the date that the Outside Director is first appointed or elected to the Board of Directors (the "Initial Stock Options" and, together with the Annual Stock Options, the "Outside Director Stock Options").

3. Terms of Outside Director Stock Options

Unless otherwise specified by the Board of Directors or the Compensation Committee at the time of grant, each Outside Director Stock Option shall: (i) vest, in the case of (A) an Annual Stock Option, on the earlier of (a) one year from the date of the grant or (b) the day prior to the annual meeting for the next fiscal year that begins following the date of grant, subject to the Outside Director's continued service on the Board of Directors on the vesting date, and (B) an Initial Stock Option, in equal annual installments over three years from the date of grant; provided that each Initial Stock Option shall in any case be fully

vested on the day prior to the annual meeting for the third fiscal year that begins following the date of grant, subject to the Outside Director's continued service on the Board of Directors on the applicable vesting dates; (ii) terminate 10 years from the date of grant, (iii) become fully vested immediately prior to a Change of Control (as defined in the Equity Plan, as amended from time to time), and (iv) be granted under the Company's standard form of agreement unless on or prior to the date of grant the Board of Directors or the Compensation Committee shall determine that other terms or conditions shall be applicable.

B. Cash Fees

1. Annual Cash Fees

The following annual cash fees shall be paid to the Outside Directors serving on the Board of Directors and the Audit Committee, Compensation Committee and Nominating and Governance Committee, as applicable.

Board of Directors or Committee of Board of Directors	Annual Retainer Amount for Chair	Annual Retainer Amount for Other Members
Board of Directors	$ 75,000	$ 40,000
Audit Committee	$ 20,000	$ 10,000
Compensation Committee	$ 15,000	$ 7,500
Nominating and Governance Committee	$ 10,000	$ 5,000

2. Payment Terms for All Cash Fees

Cash fees payable to Outside Directors shall be paid quarterly in arrears as soon as practicable following the last business day of each fiscal quarter.

Following an Outside Director's first election or appointment to the Board of Directors, such Outside Director shall receive his or her cash compensation prorated during the first fiscal quarter in which he or she was initially appointed or elected for the number of days during which he or she provides service. If an Outside Director dies, resigns or is removed during any quarter, he or she shall be entitled to a cash payment on a prorated basis through his or her last day of service that shall be paid as soon as practicable following the last business day of the fiscal quarter.

Expenses

Upon presentation of documentation of such expenses reasonably satisfactory to the Company, each Outside Director shall be reimbursed for his or her reasonable out-of-pocket business expenses incurred in connection with attending meetings of the Board of Directors and Committees thereof or in connection with other business related to the Board of Directors. Each Outside Director shall abide by the Company's travel and other expense policies applicable to Company personnel.

Amendments

The Compensation Committee or the Board of Directors shall review this Policy from time to time to assess whether any amendments in the type and amount of compensation provided herein should be adjusted in order to fulfill the objectives of this Policy.

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: May 6, 2021

/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: May 6, 2021

/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 March 31, 2021 (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: May 6, 2021

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

Dated: May 6, 2021

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