

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

FORM 10-Q

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

For the quarterly period ended March 31, 2019

or

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

For the transition period from _____ to _____
Commission File Number: 001-36257

RETROPHIN, INC.

(Exact name of registrant as specified in its charter)

Delaware

(State or other jurisdiction of incorporation or organization)

27-4842691

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

3721 Valley Centre Drive, Suite 200, San Diego, CA 92130

(Address of Principal Executive Offices)

(760) 260-8600

(Registrant's Telephone number including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.0001 per share	RTRX	The Nasdaq Global 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	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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

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

The number of shares of outstanding common stock, par value \$0.0001 per share, of the Registrant as of May 6, 2019 was 41,439,478.

RETROPHIN, INC.

Form 10-Q
For the Fiscal Quarter Ended March 31, 2019

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FORWARD LOOKING STATEMENTS

This report contains forward-looking statements regarding our business, financial condition, results of operations and prospects. Words such as “expects,” “anticipates,” “intends,” “plans,” “believes,” “seeks,” “estimates” and similar expressions or variations of such words are intended to identify forward-looking statements, but are not deemed to represent an all-inclusive means of identifying forward-looking statements as denoted in this report. Additionally, statements concerning future matters are forward-looking statements.

Although forward-looking statements in this report reflect the good faith judgment of our management, such statements can only be based on facts and factors currently known by us. Consequently, forward-looking statements are inherently subject to risks and uncertainties and actual results and outcomes may differ materially from the results and outcomes discussed in or anticipated by the forward-looking statements. Factors that could cause or contribute to such differences in results and outcomes include, without limitation, those specifically addressed under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our annual report on Form 10-K for the fiscal year ended December 31, 2018 (the “2018 10-K”), and in this quarterly report on Form 10-Q and information contained in other reports that we file with the Securities and Exchange Commission (the “SEC”). You are urged not to place undue reliance on these forward-looking statements, which speak only as of the date of this report.

We file reports with the SEC. The SEC maintains a website (www.sec.gov) that contains reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC, including us. You can also read and copy any materials we file with the SEC at the SEC’s Public Reference Room at 100 F Street, NE, Washington, DC 20549. You can obtain additional information about the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330.

We undertake no obligation to revise or update any forward-looking statements in order to reflect any event or circumstance that may arise after the date of this report, except as required by law. Readers are urged to carefully review and consider the various disclosures made throughout the entirety of this quarterly report, which are designed to advise interested parties of the risks and factors that may affect our business, financial condition, results of operations and prospects.

PART I - FINANCIAL INFORMATION**Item 1. Financial Statements**

RETROPHIN, INC. AND SUBSIDIARIES
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share amounts)

	March 31, 2019 (unaudited)	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 69,838	\$ 102,873
Marketable securities	377,808	368,668
Accounts receivable, net	12,713	12,662
Inventory, net	5,571	5,619
Prepaid expenses and other current assets	8,171	4,140
Prepaid taxes	1,412	1,716
Total current assets	475,513	495,678
Property and equipment, net	2,995	3,146
Other non-current assets	13,471	7,709
Investment-equity	15,000	15,000
Intangible assets, net	159,753	186,691
Goodwill	936	936
Total assets	\$ 667,668	\$ 709,160
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 7,627	\$ 6,954
Accrued expenses	46,619	49,695
Other current liabilities	8,292	6,165
Business combination-related contingent consideration	19,000	19,350
2019 Convertible debt	22,537	22,457
Total current liabilities	104,075	104,621
2025 Convertible debt	197,470	195,091
Other non-current liabilities	23,412	17,545
Business combination-related contingent consideration, less current portion	57,000	73,650
Total liabilities	381,957	390,907
Stockholders' Equity:		
Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2019 and December 31, 2018	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 41,438,020 and 41,389,524 issued and outstanding as of March 31, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	596,644	589,795
Accumulated deficit	(310,994)	(270,017)
Accumulated other comprehensive income (loss)	57	(1,529)
Total stockholders' equity	285,711	318,253
Total liabilities and stockholders' equity	\$ 667,668	\$ 709,160

The accompanying notes are an integral part of these condensed consolidated financial statements.

RETROPHIN, INC. AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2019	2018
Net product sales	\$ 39,570	\$ 38,432
Operating expenses:		
Cost of goods sold	1,017	1,613
Research and development	33,443	24,636
Selling, general and administrative	32,669	26,468
Change in fair value of contingent consideration	3,169	3,627
Impairment of L-UDCA IPR&D intangible asset	25,500	—
Write off of L-UDCA contingent consideration	(18,000)	—
Total operating expenses	77,798	56,344
Operating loss	(38,228)	(17,912)
Other income (expenses), net:		
Other income (expense), net	(302)	121
Interest income	2,819	797
Interest expense	(4,865)	(1,155)
Total other expense, net	(2,348)	(237)
Loss before income taxes	(40,576)	(18,149)
Income tax expense	(401)	(229)
Net loss	\$ (40,977)	\$ (18,378)
Basic and diluted net loss per common share:	\$ (0.99)	\$ (0.46)
Basic and diluted weighted average common shares outstanding:	41,410,314	39,657,418
Comprehensive loss:		
Net loss	\$ (40,977)	\$ (18,378)
Foreign currency translation	117	22
Unrealized gain (loss) on marketable securities	1,469	(536)
Comprehensive loss	\$ (39,391)	\$ (18,892)

The accompanying notes are an integral part of these condensed consolidated financial statements.

RETROPHIN, INC. AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited, in thousands)

	For the Three Months Ended March 31,	
	2019	2018
Cash Flows From Operating Activities:		
Net loss	\$ (40,977)	\$ (18,378)
Adjustments to reconcile net income to net cash used in operating activities:		
Depreciation and amortization	4,901	4,348
Non-cash interest expense	350	413
Amortization of premiums on marketable securities	(343)	356
Amortization of debt discount and issuance costs	2,459	—
Provision for Inventory	189	816
Share based compensation	6,271	4,659
Change in fair value of contingent consideration	(14,831)	3,627
Payments related to change in fair value of contingent consideration	(1,405)	(4,245)
Impairment of IPR&D intangible assets	25,500	—
Other	62	75
Changes in operating assets and liabilities, net of business acquisitions:		
Accounts receivable	40	886
Inventory	(170)	(593)
Other current and non-current operating assets	(9,321)	421
Accounts payable and accrued expenses	(3,103)	(9,330)
Other current and non-current operating liabilities	9,886	2,211
Net cash used in operating activities	(20,492)	(14,734)
Cash Flows From Investing Activities:		
Purchase of fixed assets	(19)	(39)
Cash paid for intangible assets	(3,961)	(8,217)
Proceeds from the sale/maturity of marketable securities	72,990	26,924
Purchase of marketable securities	(80,422)	(29,519)
Cash paid for investments - equity	—	(10,000)
Net cash used in investing activities	(11,412)	(20,851)
Cash Flows From Financing Activities:		
Payment of acquisition-related contingent consideration	(905)	(7,066)
Payment of guaranteed minimum royalty	(509)	(500)
Proceeds from exercise of warrants	—	608
Proceeds from exercise of stock options	304	4,256
Net cash used in financing activities	(1,110)	(2,702)
Effect of exchange rate changes on cash	(21)	10
Net change in cash and cash equivalents	(33,035)	(38,277)
Cash and cash equivalents, beginning of year	102,873	99,394
Cash and cash equivalents, end of period	\$ 69,838	\$ 61,117

The accompanying notes are an integral part of these condensed consolidated financial statements.

RETROPHIN, INC. AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands, except share amounts)
(unaudited)

	Common Stock		Additional Paid in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance - December 31, 2017	39,373,745	\$ 4	\$ 471,800	\$ (177,655)	\$ (1,015)	\$ 293,134
Adoption of ASU 2017-11 - reclassification of derivative liability of warrants with down round provisions	—	—	5,394	10,316	—	15,710
Share based compensation	—	—	4,543	—	—	4,543
Issuance of common stock from stock option exercises and vesting of restricted stock units	330,928	—	4,256	—	—	4,256
Exercise of warrants	168,612	—	608	—	—	608
Unrealized loss on marketable securities	—	—	—	—	(536)	(536)
Foreign currency translation adjustments	—	—	—	—	22	22
ESPP expense	—	—	116	—	—	116
Net loss	—	—	—	(18,378)	—	(18,378)
Balance - March 31, 2018	39,873,285	\$ 4	\$ 486,717	\$ (185,717)	\$ (1,529)	\$ 299,475
Balance - December 31, 2018	41,389,524	\$ 4	\$ 589,795	\$ (270,017)	\$ (1,529)	\$ 318,253
Share based compensation	—	—	6,370	—	—	6,370
Issuance of common shares under the equity incentive plan and proceeds from exercise	48,496	—	304	—	—	304
Unrealized gain on marketable securities	—	—	—	—	1,469	1,469
Foreign currency translation adjustments	—	—	—	—	117	117
ESPP expense	—	—	175	—	—	175
Net loss	—	—	—	(40,977)	—	(40,977)
Balance - March 31, 2019	41,438,020	\$ 4	\$ 596,644	\$ (310,994)	\$ 57	\$ 285,711

The accompanying notes are an integral part of these condensed consolidated financial statements.

RETROPHIN, INC. AND SUBSIDIARIES
NOTES TO THE UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1. DESCRIPTION OF BUSINESS

Organization and Description of Business

Retrophin, Inc. (“we”, “our”, “us”, “Retrophin” and the “Company”) refers to Retrophin, Inc., a Delaware corporation, as well as our direct and indirect subsidiaries. Retrophin is a biopharmaceutical company headquartered in San Diego, California, focused on identifying, developing and delivering life-changing therapies to people living with rare diseases. We regularly evaluate and, where appropriate, act on opportunities to expand our product pipeline through licenses and acquisitions of products in areas that will serve patients with rare diseases and that we believe offer attractive growth characteristics.

The Company is developing the following pipeline products:

The Company is developing fosmetpantotenate (RE-024), a novel small molecule, as a potential treatment for pantothenate kinase-associated neurodegeneration (“PKAN”). PKAN is a genetic neurodegenerative disorder that is typically diagnosed in the first decade of life.

Sparsentan, also known as RE-021, is an investigational product candidate with a dual mechanism of action, a potent angiotensin receptor blocker (“ARB”) and selective endothelin receptor antagonist (“ERA”), with in vitro selectivity toward endothelin receptor type A. Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in the following indications:

- Focal segmental glomerulosclerosis (“FSGS”) is a rare kidney disease characterized by proteinuria where the glomeruli become progressively scarred. FSGS is a leading cause of end-stage renal disease.
- Immunoglobulin A nephropathy (“IgAN”) is an immune-complex-mediated glomerulonephritis characterized by hematuria, proteinuria, and variable rates of progressive renal failure. IgAN is the most common primary glomerular disease.

The Company is a party to a joint development agreement with Censa Pharmaceuticals Inc. (“Censa”), a privately held biotechnology company focused on developing therapies for orphan metabolic diseases, to evaluate sepiapterin (“CNSA-001”) for the treatment of phenylketonuria (PKU).

In September 2017, the Company entered into a three-way Cooperative Research and Development Agreement (“CRADA”) with the National Institutes of Health’s National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency.

The Company sells the following three products:

- Chenodal (chenodiol tablets) is approved in the United States for the treatment of patients suffering from gallstones in whom surgery poses an unacceptable health risk due to disease or advanced age. Chenodal has been the standard of care for cerebrotendinous xanthomatosis (“CTX”) patients for more than three decades and the Company is currently pursuing adding this indication to the label.
- Cholbam (cholic acid capsules) is approved in the United States for the treatment of bile acid synthesis disorders due to single enzyme defects and is further indicated for adjunctive treatment of patients with peroxisomal disorders.
- Thiola (tiopronin tablets) is approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.

NOTE 2. BASIS OF PRESENTATION AND SIGNIFICANT ACCOUNTING POLICIES

The accompanying unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements and notes thereto included in the 2018 10-K filed with the SEC on February 26, 2019. The accompanying condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States (“GAAP”) for interim financial information, the instructions for Form 10-Q and the rules and regulations of the SEC. Accordingly, since they are interim statements, the accompanying condensed consolidated financial statements do not include all of the information and notes required by GAAP for annual financial statements, but reflect all adjustments consisting of normal, recurring adjustments, that are necessary for a fair presentation of the financial position, results of operations and cash flows for the interim periods presented. Interim results are not necessarily indicative of the results that may be expected for any future periods. The December 31, 2018 balance sheet information was derived from the audited financial statements as of that date. Certain reclassifications have been made to the prior period consolidated financial statements to conform to the current period presentation.

A summary of the significant accounting policies applied in the preparation of the accompanying condensed consolidated financial statements follows:

Principles of Consolidation

The unaudited condensed consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with GAAP. All intercompany accounts and transactions have been eliminated in consolidation.

Revenue Recognition

The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of Topic 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect substantially all the consideration it is entitled to in exchange for the goods or services it transfers to the customer. See Note 3 for further discussion.

Research and Development Expenses

Research and development expenses are comprised of salaries and bonuses, benefits, non-cash share-based compensation, license fees, costs paid to third-party contractors to perform research, conduct clinical trials and pre/non-clinical trials, develop drug materials, and associated overhead expenses and facilities. We also incur indirect costs that are not allocated to specific programs because such costs benefit multiple development programs and allow us to increase our pharmaceutical development capabilities. These consist of internal shared resources related to the development and maintenance of systems and processes applicable to all of our programs.

Clinical Trial Expenses

Our clinical trials are conducted pursuant to contracts with contract research organizations (CROs) that support conducting and managing clinical trials. The financial terms and activities of these agreements vary from contract to contract and may result in uneven expense levels. Generally, these agreements set forth activities that drive the recording of expenses such as start-up, initiation activities, enrollment, treatment of patients, or the completion of other clinical trial activities.

Expenses related to clinical trials are accrued based on our estimates and/or representations from service providers regarding work performed, including actual level of patient enrollment, progress of the clinical trials, and completion of patient studies. Other incidental costs related to patient enrollment or treatment are accrued when reasonably certain. If the amounts we are obligated to pay under our clinical trial agreements are modified (for instance, as a result of changes in the clinical trial protocol or scope of work to be performed), we adjust our accruals accordingly on a prospective basis. Revisions to our contractual payment obligations are charged to expense in the period in which the facts that give rise to the revision become reasonably certain.

We currently have three Phase 3 clinical trials in process that are in varying stages of activity, with ongoing non-clinical support trials. As such, clinical trial expenses will vary depending on all the factors set forth above and may fluctuate significantly from quarter to quarter.

Adoption of New Accounting Standards

In February 2016, the FASB issued ASU No. 2016-02, Leases. The new standard establishes a right-of-use ("ROU") model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. The new standard is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. See Note 6 for further discussion.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies. Unless otherwise noted, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its consolidated financial position or results of operations upon adoption.

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments. Topic 326 amends guidance on reporting credit losses for assets held at amortized cost basis and available for sale debt securities. For assets held at amortized cost basis, Topic 326 eliminates the probable initial recognition threshold in current GAAP and, instead, requires an entity to reflect its current estimate of all expected credit losses. The allowance for credit losses is a valuation account that is deducted from the amortized cost basis of the financial assets to present the net amount expected to be collected. For available for sale debt securities, credit losses should be measured in a manner similar to current GAAP, however Topic 326 will require that credit losses be presented as an allowance rather than as a write-down. This ASU update affects entities holding financial assets and net investment in leases that are not accounted for at fair value through net income. The amendments affect loans, debt securities, trade receivables, net investments in leases, off balance sheet credit exposures, reinsurance receivables, and any other financial assets not excluded from the scope that have the contractual right to receive cash. This update is effective for fiscal years beginning after December 15, 2019, including interim periods within those fiscal years. As of March 31, 2019, the Company held \$377.8 million in available for sale debt securities. If adopted as of March 31, 2019, this ASU update would not have a material impact on the Company's financial statements.

NOTE 3. REVENUE RECOGNITION

Product Revenue, Net

The Company sells Chenodal and Cholbam (Kolbam), which are aggregated as bile acid products, and Thiola through direct-to-patient distributors. The Company sells its products worldwide, with more than 95% of the revenue generated in North America.

Revenues from product sales are recognized when the customer obtains control of the Company's product, which occurs upon delivery to the customer.

Deductions from Revenue

Revenues from product sales are recorded at the net sales price, which includes provisions resulting from discounts, rebates and co-pay assistance that are offered to its customers, health care providers, payors and other indirect customers relating to the Company's sales of its products. These provisions are based on the amounts earned or to be claimed on the related sales and are classified as a reduction of accounts receivable (if the amount is payable to the customer) or as a current liability (if the amount is payable to a party other than a customer). Where appropriate, these reserves take into consideration the Company's historical experience, current contractual and statutory requirements, specific known market events and trends, industry data and forecasted customer buying and payment patterns. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contract. If actual results in the future vary from the Company's provisions, the Company will adjust the provision, which would affect net product revenue and earnings in the period such variances become known. Our historical experience is that such adjustments have been immaterial.

Government Rebates: We calculate the rebates that we will be obligated to provide to government programs and deduct these estimated amounts from our gross product sales at the time the revenues are recognized. Allowances for government rebates and discounts are established based on actual payer information, which is reasonably estimated at the time of delivery, and the government-mandated discounts applicable to government-funded programs. Rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets.

Commercial Rebates: We calculate the rebates that we incur due to contracts with certain commercial payors and deduct these amounts from our gross product sales at the time the revenues are recognized. Allowances for commercial rebates are established based on actual payer information, which is reasonably estimated at the time of delivery. Rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets.

Prompt Pay Discounts: We offer discounts to certain customers for prompt payments. We accrue for the calculated prompt pay discount based on the gross amount of each invoice for those customers at the time of sale.

Product Returns: Consistent with industry practice, we offer our customers a limited right to return product purchased directly from the Company, which is principally based upon the product's expiration date. Generally, shipments are only made upon a patient prescription thus returns are minimal.

Co-pay Assistance: We offer a co-pay assistance program, which is intended to provide financial assistance to qualified commercially insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an identification of claims and the cost per claim associated with product that has been recognized as revenue.

The following table summarizes net product revenues for the three months ended March 31, 2019 and 2018 (*in thousands*):

	Three Months Ended March 31,	
	2019	2018
Bile acid products	\$ 18,390	\$ 18,508
Thiola	21,180	19,924
Total net product revenue	<u>\$ 39,570</u>	<u>\$ 38,432</u>

NOTE 4. MARKETABLE SECURITIES

The Company's marketable securities as of March 31, 2019 and December 31, 2018 were comprised of available-for-sale marketable securities which are carried at fair value, with the unrealized gains and losses reported in accumulated other comprehensive income. Realized gains and losses and declines in value judged to be other-than-temporary, if any, on available-for-sale securities are included in other income or expense. Interest and dividends on securities classified as available-for-sale are included in interest income. The amortized cost of debt securities is adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion is included in interest income. During the three months ended March 31, 2019, investment activity for the Company included \$73.0 million in maturities and \$80.4 million in purchases, all relating to debt based marketable securities.

Marketable securities consisted of the following (in thousands):

	March 31, 2019	December 31, 2018
Commercial paper	\$ 53,373	\$ 59,255
Corporate debt securities	324,435	299,413
Securities of government sponsored entities	—	10,000
Total marketable securities:	<u>\$ 377,808</u>	<u>\$ 368,668</u>

The following is a summary of short-term marketable securities classified as available-for-sale as of March 31, 2019 (in thousands):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Commercial paper	Less than 1	\$ 53,366	\$ 20	\$ (13)	\$ 53,373
Corporate debt securities	Less than 1	147,633	28	(200)	147,461
Total maturity less than 1 year		<u>200,999</u>	<u>48</u>	<u>(213)</u>	<u>200,834</u>
Corporate debt securities	1 to 2	176,622	436	(84)	176,974
Total maturity 1 to 2 years		<u>176,622</u>	<u>436</u>	<u>(84)</u>	<u>176,974</u>
Total available-for-sale securities		<u>\$ 377,621</u>	<u>\$ 484</u>	<u>\$ (297)</u>	<u>\$ 377,808</u>

The following is a summary of short-term marketable securities classified as available-for-sale as of December 31, 2018 (in thousands):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Commercial paper	Less than 1	\$ 59,313	\$ —	\$ (58)	\$ 59,255
Corporate debt securities	Less than 1	149,824	—	(604)	149,220
Total maturity less than 1 year		<u>209,137</u>	<u>—</u>	<u>(662)</u>	<u>208,475</u>
Corporate debt securities	1 to 2	150,813	18	(638)	150,193
Securities of government-sponsored entities	1 to 2	9,997	4	(1)	10,000
Total maturity 1 to 2 years		<u>160,810</u>	<u>22</u>	<u>(639)</u>	<u>160,193</u>
Total available-for-sale securities		<u>\$ 369,947</u>	<u>\$ 22</u>	<u>\$ (1,301)</u>	<u>\$ 368,668</u>

The primary objective of the Company's investment portfolio is to enhance overall returns while preserving capital and liquidity. The Company's investment policy limits interest-bearing security investments to certain types of instruments issued by institutions with primarily investment grade credit ratings and places restrictions on maturities and concentration by asset class and issuer. All available for sale securities are held in current assets regardless of contractual maturities exceeding one year, as the Company has the ability to sell them within the next twelve months.

The Company reviews the available-for-sale investments for other-than-temporary declines in fair value below cost basis each quarter and whenever events or changes in circumstances indicate that the cost basis of an asset may not be recoverable. This evaluation is based on a number of factors, including the length of time and the extent to which the fair value has been below the cost basis and adverse conditions related specifically to the security, including any changes to the credit rating of the security, and the intent to sell, or whether the Company will more likely than not be required to sell the security before recovery of its amortized cost basis. The assessment of whether a security is other-than-temporarily impaired could change in the future due to new developments or changes in assumptions related to any particular security. As of March 31, 2019 and December 31, 2018, the Company believed the cost basis for available-for-sale investments was recoverable in all material respects.

NOTE 5. FUTURE ACQUISITION RIGHT AND JOINT DEVELOPMENT AGREEMENT

Censa Pharmaceuticals Inc.

In December 2017, the Company entered into a Future Acquisition Right and Joint Development Agreement (the "Option Agreement") with Censa, which became effective in January 2018. The Company made an upfront payment of \$10 million, agreed to fund certain development activities of Censa's CNSA-001 program which are expected to be approximately \$19.7 million through proof of concept, and paid \$5.0 million related to a development milestone for the right, but not the obligation, to acquire Censa (the "Option") on the terms and subject to the conditions set forth in a separate Agreement and Plan of Merger. The Company capitalized the upfront and milestone payments and has expensed the development funding as incurred. The Company is treating the upfront payment and milestone payment, both of which are consideration for the Option, as a cost-method investment with a carrying value of \$15.0 million as of March 31, 2019.

If the Company exercises the Option, the Company will acquire Censa for an additional \$65 million, which may be reduced by up to \$2.6 million of development funding ("creditable"), paid as a combination of 20% in cash and 80% in shares of the Company's common stock, valued at a fixed price of \$21.40 per share; provided, however, that Censa may elect on behalf of its equity holders to receive the upfront consideration in 100% cash

if the average price per share of the Company's common stock for the ten trading days ending on the date the Company provides a notice of interest to exercise the Option is less than \$19.26. In addition, if the Company exercises the Option and acquires Censa, the Company would be required to make further cash payments to Censa's equity holders of up to an aggregate of \$25 million if the CNSA-001 program achieves specified development and commercial milestones.

The Company determined that Censa is a variable interest entity ("VIE"), however concluded that the Company is not the primary beneficiary of the VIE. As such, the Company did not consolidate Censa's results into its consolidated financial statements. The Company will continue to monitor facts and circumstances for changes that could potentially result in the Company becoming the primary beneficiary.

The following table presents the Company's development funding roll-forward through March 31, 2019 and its effect on the upfront consideration if the Option exercise is elected (*in thousands*):

	March 31, 2019
Non-creditable development funding commitment	\$ 17,091
Development funding creditable against purchase option	2,612
Total development funding	19,703
Development funding paid through March 31, 2019	18,299
Development funding payable	\$ 1,404

NOTE 6. LEASES

As of January 1, 2019 the Company adopted ASU No. 2016-02, Leases, using a modified retrospective basis method under which prior comparative periods are not restated.

The new standard establishes a ROU model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. In addition, the FASB issued ASU No. 2018-10, Codification Improvements to Topic 842, ASU No. 2018-11, Targeted Improvements, and ASU No. 2018-20, Narrow-Scope Improvements for Lessors, to clarify and amend the guidance in ASU No. 2016-02. The Company has elected the following as practical expedients from within these ASUs: 1) an entity need not reassess whether any expired or existing contracts are or contain leases, 2) an entity need not reassess the lease classification for any expired or existing leases, and 3) an entity need not reassess initial direct costs for any existing leases.

As of January 1, 2019 the Company had a single operating lease for its office located in San Diego, CA. The lease was originally signed in July 2016, amended in July 2017, and is for approximately 45,000 square feet of office space in adjacent buildings. The term of the original lease is 7 years, 7 months, and is coterminous for all space occurring in July 2024. Under the terms of the lease, the Company will pay base annual rent (subject to an annual fixed percentage increase), plus property taxes and other normal and necessary expenses, such as utilities, repairs, security and maintenance. Certain incentives were included in the lease, including approximately \$2.3 million in tenant improvement allowances and seven months of rent abatement. The Company has the right to extend the lease for five years.

As of January 1, 2019 the Company's remaining minimum lease payments and unamortized lease incentives were approximately \$14 million and \$1.8 million, respectively. Using a discount rate equal to our borrowing rate of 7.7% and a remaining term of 5 years, 7 months, the Company determined the ROU asset and lease liability as of adoption were \$7.9 million and \$11.3 million, respectively. There was no cumulative adjustment to our beginning accumulated deficit balance.

In March 2019, the company amended the existing office lease to add approximately 16,000 square feet of office space in adjacent buildings. The additional space is expected to be utilized through August 2020 and has future minimum lease payments of approximately \$1.0 million. The Company determined the ROU asset and lease liability were each \$1.0 million.

Following is a schedule of the future minimum rental commitments for our operating lease reconciled to the lease liability and ROU assets as of March 31, 2019 (*in thousands*):

	March 31, 2019
2019	\$ 2,250
2020	2,958
2021	2,486
2022	2,560
2023	2,637
Thereafter	1,585
Total undiscounted future minimum payments	14,476
Present value discount	(2,530)
Total lease liability	11,946
Lease incentives	(1,702)
Straight line lease expense in excess of cash payments	(1,635)
Total ROU asset	\$ 8,609

As of March 31, 2019, the current and non-current portions of the ROU asset were recorded to the Condensed Consolidated Balance Sheet as follows (*in thousands*):

	March 31, 2019
Prepaid expenses and other current assets	\$ 2,590
Other non-current assets	6,019
Total ROU asset	\$ 8,609

As of March 31, 2019, the current and non-current portions of the lease liability were recorded to the Condensed Consolidated Balance Sheet as follows (*in thousands*):

	March 31, 2019
Other current liabilities	\$ 3,054
Other non-current liabilities	8,892
Total lease liabilities	\$ 11,946

For the 3 months ended March 31, 2019, the Company recorded \$0.7 million in expense related to operating leases .

NOTE 7. FAIR VALUE MEASUREMENTS

Financial Instruments and Fair Value

The Company accounts for financial instruments in accordance with ASC 820, *Fair Value Measurements and Disclosures* (“ASC 820”). ASC 820 establishes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurements) and the lowest priority to unobservable inputs (Level 3 measurements). The three levels of the fair value hierarchy under ASC 820 are described below:

Level 1 – Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;

Level 2 – Quoted prices in markets that are not active or financial instruments for which all significant inputs are observable, either directly or indirectly; and

Level 3 – Prices or valuations that require inputs that are both significant to the fair value measurement and unobservable.

The valuation techniques used to measure the fair value of the Company’s marketable securities and all other financial instruments, all of which have counter-parties with high credit ratings, were valued based on quoted market prices or model driven valuations using significant inputs derived from or corroborated by observable market data. Based on the fair value hierarchy, the Company classified marketable securities within Level 2.

In estimating the fair value of the Company’s contingent consideration, the Company used the Monte Carlo Simulation model as of March 31, 2019 and December 31, 2018. Based on the fair value hierarchy, the Company classified the fair value measurement of contingent consideration within Level 3.

Financial instruments with carrying values approximating fair value include cash and cash equivalents, accounts receivable, and accounts payable, due to their short term nature. As of March 31, 2019, the estimated fair value of the Company’s 4.5% Senior Convertible Notes due 2019 was \$29.7

million and the estimated fair value of the Company's 2.5% Convertible Senior Notes due 2025 was \$262.4 million, considering factors such as market conditions, prepayment and make-whole provisions, variability in pricing from multiple lenders and the term of the debt.

The following table presents the Company's assets and liabilities, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of March 31, 2019 (*in thousands*):

	As of March 31, 2019			
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
Cash and Cash Equivalents	\$ 69,838	\$ 49,173	\$ 20,665	—
Marketable securities, available-for-sale	377,808	—	377,808	—
Total	\$ 447,646	\$ 49,173	\$ 398,473	\$ —
Liabilities:				
Business combination-related contingent consideration	\$ 76,000	\$ —	\$ —	\$ 76,000
Total	\$ 76,000	\$ —	\$ —	\$ 76,000

The following table presents the Company's assets and liabilities, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of December 31, 2018 (*in thousands*):

	As of December 31, 2018			
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
Cash and Cash Equivalents	\$ 102,873	\$ 62,978	\$ 39,895	\$ —
Marketable securities, available-for-sale	368,668	—	368,668	—
Total	\$ 471,541	\$ 62,978	\$ 408,563	\$ —
Liabilities:				
Business combination-related contingent consideration	93,000	—	—	93,000
Total	\$ 93,000	\$ —	\$ —	\$ 93,000

The following table sets forth a summary of changes in the estimated fair value of the Company's business combination-related contingent consideration for the three months ended March 31, 2019 (*in thousands*):

	Fair Value Measurements of Acquisition-Related Contingent Consideration (Level 3)
Balance at January 1, 2019	\$ 93,000
L-UDCA write-off	(18,000)
Changes in the fair value of contingent consideration	3,169
Contractual payments included in accrued liabilities at March 31, 2019	(2,093)
Contractual payments	—
Foreign currency impact	(76)
Balance at March 31, 2019	\$ 76,000

The key assumptions included in the calculations for contingent consideration were the future number of patients in treatment, projected revenues, discount rate, and the timing of payments. The present value of the expected payments considers the time at which the obligations are expected to be settled and a discount rate that reflects the risk associated with the performance payments.

During the three months ended March 31, 2019, the Company recognized \$3.2 million in operating expense on the Condensed Consolidated Statement of Operations and Comprehensive Loss for the change in fair value of the contingent consideration liabilities. For the three months ended March 31, 2019, \$1.5 million and \$1.6 million of the charges were related to the increase in contingent consideration liabilities for the products Chenodal and Cholbam, respectively. In each case, the value increased due to passage of time. In addition, the Company made a portfolio decision not to pursue further development of L-UDCA. The related contingent consideration of \$18.0 million was accordingly considered fully written off. See Note 17 for further discussion.

During the three months ended March 31, 2018, the Company incurred charges of \$3.6 million in operating expenses on the Condensed Consolidated Statement of Operations and Comprehensive Loss for the change in fair value of the contingent consideration liabilities. For the three months ended March 31, 2018, \$0.8 million, \$1.8 million, and \$1.0 million of the charges were related to the increase in contingent consideration

liabilities for the products Chenodal and Cholbam and product candidate L-UDCA, respectively. In each case, the value increased due to passage of time.

NOTE 8. INTANGIBLE ASSETS

As of March 31, 2019, the net book value of amortizable intangible assets was approximately \$159.8 million.

The following table sets forth amortizable intangible assets as of March 31, 2019 and December 31, 2018 (*in thousands*):

	March 31, 2019	December 31, 2018
Finite-lived intangible assets	\$ 233,375	\$ 255,643
Less: accumulated amortization	(73,622)	(68,952)
Net carrying value	<u>\$ 159,753</u>	<u>\$ 186,691</u>

During the three months ended March 31, 2019, the Company made a portfolio decision not to pursue further development of L-UDCA, acquired in 2016. The related in-progress research and development intangible asset ("IPR&D") of \$25.5 million was accordingly considered fully impaired and written off. As of December 31, 2018, the value of the IPR&D was \$25.5 million. See Note 17 for further discussion.

The following table summarizes amortization expense for the three months ended March 31, 2019 and 2018 (*in thousands*):

	Three Months Ended March 31,	
	2019	2018
Research and development	\$ 286	\$ 103
Selling, general and administrative	4,445	4,096
Total amortization expense	<u>\$ 4,731</u>	<u>\$ 4,199</u>

NOTE 9. CONVERTIBLE NOTES PAYABLE

Convertible Senior Notes Due 2025

On September 10, 2018, the Company completed its registered underwritten public offering of \$276 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes"), and entered into a base indenture and supplemental indenture agreement ("2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025 ("Maturity Date"), unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year. The first payment was made on March 15, 2019.

The composition of the Company's 2025 Notes are as follows (*in thousands*):

	March 31, 2019	December 31, 2018
2.50% convertible senior notes due 2025	\$ 276,000	\$ 276,000
Unamortized debt discount	(72,681)	(74,836)
Unamortized debt issuance costs	(5,849)	(6,073)
Total 2025 Notes, net of unamortized debt discount and debt issuance costs	<u>\$ 197,470</u>	<u>\$ 195,091</u>

The net proceeds from the issuance of the 2025 Notes were approximately \$267.2 million, after deducting commissions and the offering expenses payable by the Company. A portion of the net proceeds from the 2025 Notes were used by the Company to repurchase \$23.4 million aggregate principal amount of its then-outstanding 4.5% senior convertible notes due in 2019 in privately-negotiated transactions.

Holders may convert their 2025 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on December 31, 2018 (and only during such calendar quarter), if the last reported sale price per share of the Company's common stock for each of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period ("measurement period") if the trading price per \$1,000 principal amount of 2025 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of the Company's common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain corporate events or distributions on the Company's common stock; (4) if the Company calls the 2025 Notes for redemption; and (5) at any time from, and including, May 15, 2025 until the close of business on the scheduled trading day immediately before the Maturity Date. The Company will settle conversions by paying or delivering, as applicable, cash, shares of the Company's common stock, or a combination of cash and shares of the Company's common stock, at the Company's election, based on the applicable conversion rate.

The initial conversion rate for the 2025 Notes is 25.7739 shares of the Company’s common stock per \$1,000 principal amount of 2025 Notes, which represents an initial conversion price of approximately \$38.80 per share. If a “make-whole fundamental change” (as defined in the 2025 Indenture) occurs, then the company will, in certain circumstances, increase the conversion rate for a specified period of time.

The 2025 Notes will be redeemable, in whole or in part, at the Company’s option at any time, and from time to time, on or after September 15, 2022 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the Maturity Date, at a cash redemption price equal to the principal amount of the 2025 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date, but only if the last reported sale price per share of the Company’s common stock exceeds 130% of the conversion price on each of at least 20 trading days during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related redemption notice. If a fundamental change (as defined in the 2025 Indenture) occurs, then, subject to certain exceptions, holders may require the Company to repurchase their 2025 Notes at a cash repurchase price equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date.

In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2025 Notes will be paid pursuant to the terms of the 2025 Indenture. In the event that all of the 2025 Notes are converted, the Company would be required to repay the \$276.0 million in principal value and any conversion premium in any combination of cash and shares of its common stock at the Company’s option. In addition, calling the 2025 Notes for redemption will constitute a “make whole fundamental change.”

The 2025 Notes are the Company’s general unsecured obligations that rank senior in right of payment to all of its indebtedness that is expressly subordinated in right of payment to the 2025 Notes, and equal in right of payment to the Company’s unsecured indebtedness.

The 2025 Notes are currently classified on the Company’s consolidated balance sheet at March 31, 2019 as long-term debt.

Under ASC 470-20, Debt with Conversion and Other Options, an entity must separately account for the liability and equity components of convertible debt instruments (such as the 2025 Notes) that may be settled entirely or partially in cash upon conversion, in a manner that reflects the issuer’s economic interest cost. The liability component of the instrument is valued in a manner that reflects the market interest rate for a similar nonconvertible instrument at the date of issuance. The initial carrying value of the liability component was \$198.6 million. The equity component of \$77.4 million, representing the conversion option, was determined by deducting the fair value of the liability component from the par value of the 2025 Notes and is recorded in additional paid-in capital on the consolidated balance sheet at the issuance date. That equity component is treated as a discount on the liability component of the 2025 Notes, which is amortized over the seven year term of the 2025 Notes using the effective interest rate method. The equity component is not re-measured as long as it continues to meet the conditions for equity classification. The Company allocated the total transaction costs of approximately \$8.8 million related to the issuance of the 2025 Notes to the liability and equity components of the 2025 Notes based on their relative values. Transaction costs attributable to the liability component are amortized to interest expense over the seven-year term of the 2025 Notes, and transaction costs attributable to the equity component are netted with the equity component in stockholders’ equity.

The effective interest rate on the liability components of the 2025 Notes for the period from the date of issuance through March 31, 2019 was 7.7%. The following table sets forth total interest expense recognized related to the 2025 Notes (*in thousands*):

	Three Months Ended March 31,		
	2019	2018	
Contractual interest expense	1,725	—	
Amortization of debt discount	2,155	—	
Amortization of debt issuance costs	224	—	
Total interest expense for the 2025 Notes	\$ 4,104	\$ —	

The 2025 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by the Company. The 2025 Indenture contains customary events of default with respect to the 2025 Notes, including that upon certain events of default, 100% of the principal and accrued and unpaid interest on the 2025 Notes will automatically become due and payable.

Senior Convertible Notes Due 2019

On May 29, 2014, the Company entered into a Note Purchase Agreement relating to a private placement by the Company of \$46.0 million aggregate principal 4.50% senior convertible notes due in 2019 (the “2019 Notes”) which are convertible into shares of the Company’s common stock at an initial conversion price of \$17.41 per share. The conversion price is subject to customary anti-dilution protection. The 2019 Notes bear interest at a rate of 4.5% per annum, payable semiannually in arrears on May 15 and November 15 of each year. The 2019 Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with their terms, and there are no contractual payments due prior to that date.

In September 2018, the Company used part of the net proceeds from the issuance of the 2025 Notes noted above to repurchase \$23.4 million aggregate principal value of the 2019 Notes in privately-negotiated transactions for approximately \$40.2 million in cash. The partial repurchase of the 2019 Notes resulted in a \$17.0 million loss on early extinguishment of debt in September 2018. At March 31, 2019, approximately \$22.6 million of principal remained outstanding on the 2019 Notes.

The composition of the Company's 2019 Notes are as follows (*in thousands*):

	March 31, 2019	December 31, 2018
4.50% senior convertible notes due 2019	\$ 22,590	\$ 22,590
Unamortized debt discount	(50)	(125)
Unamortized debt issuance costs	(3)	(8)
Total 2019 Notes, net of unamortized debt discount and debt issuance costs	<u>\$ 22,537</u>	<u>\$ 22,457</u>

NOTE 10. ACCRUED EXPENSES

Accrued expenses at March 31, 2019 and December 31, 2018 consisted of the following (*in thousands*):

	March 31, 2019	December 31, 2018
Government rebates payable	\$ 8,988	\$ 8,464
Compensation related costs	7,856	10,446
Accrued royalties and contingent consideration	6,042	6,805
Research and development	17,955	16,515
Selling, general and administrative	3,812	2,990
Miscellaneous accrued	1,966	4,475
Total accrued expenses	<u>\$ 46,619</u>	<u>\$ 49,695</u>

NOTE 11. LOSS PER COMMON SHARE

Basic and diluted net loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period, without consideration of common stock equivalents. The Company's potentially dilutive shares, which include outstanding stock options, restricted stock units, warrants, and shares issuable upon conversion of the 2019 Notes and 2025 Notes, are considered to be common stock equivalents and are only included in the calculation of diluted net loss per share when their effect is dilutive.

Basic and diluted net loss per share is calculated as follows (*net loss amounts are stated in thousands*):

	Three Months Ended March 31,					
	2019			2018		
	Shares	Net Loss	EPS	Shares	Net Loss	EPS
Basic and diluted loss per share	41,410,314	\$ (40,977)	\$ (0.99)	39,657,418	\$ (18,378)	\$ (0.46)

The following common stock equivalents have been excluded because they were anti-dilutive (*in thousands*):

	Three Months Ended March 31,	
	2019	2018
Restricted stock units	549	139
Convertible debt	8,411	2,642
Options	7,654	7,025
Warrants	—	633
Total anti-dilutive shares	<u>16,614</u>	<u>10,439</u>

NOTE 12. COMMITMENTS AND CONTINGENCIES

Research Collaboration and Licensing Agreements

As part of the Company's research and development efforts, the Company enters into research collaboration and licensing agreements with unrelated companies, scientific collaborators, universities, and consultants. These agreements contain varying terms and provisions which include fees and milestones to be paid by the Company, services to be provided, and ownership rights to certain proprietary technology developed under the agreements. Some of these agreements contain provisions which require the Company to pay royalties, in the event the Company sells or licenses any proprietary products developed under the respective agreements.

Legal Proceedings

In August 2017, Martin Shkreli, the Company's former Chief Executive Officer, was convicted on securities fraud charges following investigations by the U.S. Attorney for the Eastern District of New York and the U.S. Securities and Exchange Commission. The Company was not a target of these investigations and cooperated with them fully. Mr. Shkreli has appealed his conviction to the United States Court of Appeals for the Second Circuit. In connection with the trial and pending appeal proceedings, Mr. Shkreli sought advancement of his legal fees from the Company, and the Company has advanced a total of \$5.4 million in legal fees, of which \$3.8 million has been reimbursed by its directors' and officers' insurance carriers. Pending the outcome of Mr. Shkreli's appeal, the insurance carriers have reserved their rights to assert that certain of the advanced funds pertain to claims excluded from coverage under the relevant insurance policy and are therefore recoverable by the carriers. As a result, the final amount of the reimbursement from the insurance carriers is not currently estimable. In addition, a portion of these and the other legal fees the Company has advanced to Mr. Shkreli will be subject to reimbursement by Mr. Shkreli under Delaware law in the event it is ultimately determined that Mr. Shkreli is not entitled to be indemnified by the Company in these proceedings.

In August 2015, the Company filed a lawsuit in federal district court for the Southern District of New York against Mr. Shkreli, asserting that he breached his fiduciary duty of loyalty during his tenure as the Company's Chief Executive Officer and a member of its Board of Directors. Mr. Shkreli served a demand for JAMS arbitration on Retrophin, claiming that Retrophin had breached his December 2013 employment agreement. In response to Mr. Shkreli's arbitration demand, the Company asserted counterclaims in the arbitration that are substantially similar to the claims it previously asserted in the federal lawsuit against Mr. Shkreli. In October 2018, after the arbitration panel determined that Retrophin's counterclaims were arbitrable, the Company voluntarily dismissed the federal action without prejudice. The Company does not expect the claims and counterclaims in the arbitration to be heard by the arbitration panel before mid-2019. In connection with these proceedings, Mr. Shkreli sought advancement of his legal fees from the Company relating to his defense of the Company's claims against him. The Company has advanced, and expects to continue to advance, certain of these legal fees to Mr. Shkreli.

On October 23, 2018, Spring Pharmaceuticals, LLC (Spring) filed a lawsuit against the Company, Martin Shkreli, Mission Pharmacal Company and Alamo Pharma Services, Inc. in the United States District Court for the Eastern District of Pennsylvania alleging that the Company violated various federal and state antitrust and unfair competition laws by allegedly refusing to sell samples of the Thiola® brand drug so that Spring can conduct the bioequivalence testing needed to submit an ANDA to the FDA for approval to market a generic version of the product. Spring is seeking injunctive relief and damages. The Company intends to vigorously defend against Spring's claims. On January 15, 2019, the Company filed a motion to dismiss the lawsuit. On April 10, 2019, the Court stayed the Company's motion to dismiss for 90 days to allow for discovery limited to the question of whether Spring has standing to sue. No amounts have been accrued related to this matter and the outcome cannot be determined.

The Company is not aware of any other proceedings or claims that could have, individually or in the aggregate, a material adverse effect on its results of operations or financial condition.

NOTE 13. SHARE BASED COMPENSATION

Restricted Shares

Service Based Restricted Stock Units

The following table summarizes the Company's service based restricted stock unit activity during the three months ended March 31, 2019:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested December 31, 2018	400,426	\$ 24.95
Granted	62,850	23.12
Vested	(24,769)	20.88
Forfeited/canceled	(1,250)	25.25
Unvested March 31, 2019	437,257	\$ 24.92

At March 31, 2019, unamortized stock compensation for service based restricted stock units was \$8.3 million, with a weighted-average recognition period of 3.0 years.

Performance Based Restricted Stock Units

The following table summarizes the Company's performance based restricted stock unit activity during the three months ended March 31, 2019:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested December 31, 2018	226,750	\$ 21.54
Granted	50,000	23.34
Vested	—	—
Forfeited/canceled	—	—
Unvested March 31, 2019	276,750	\$ 21.86

At March 31, 2019, unamortized stock compensation for performance based restricted stock units was \$1.8 million, with a weighted-average recognition period of 1.2 years.

Stock Options

The following table summarizes stock option activity during the three months ended March 31, 2019:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2018	7,277,337	\$ 18.55	6.94	\$ 40,650
Granted	440,700	23.20		
Exercised	(23,727)	12.81		
Forfeited/canceled	(33,134)	24.94		
Outstanding at March 31, 2019	7,661,176	\$ 18.81	6.87	\$ 40,435

At March 31, 2019, unamortized stock compensation for stock options was \$30.9 million, with a weighted-average recognition period of 2.8 years.

At March 31, 2019, outstanding options to purchase 5.0 million shares of common stock were exercisable with a weighted-average exercise price per share of \$16.92.

Share Based Compensation

The following table sets forth total stock-based compensation for the three months ended March 31, 2019 and 2018 (in thousands):

	Three Months Ended March 31,	
	2019	2018
Research and development	\$ 1,670	\$ 1,407
Selling, general & administrative	4,850	3,202
Total	\$ 6,520	\$ 4,609

Exercise of Warrants

The Company issued 168,612 shares of common stock upon the exercise of outstanding warrants during the three months ended March 31, 2018, for cash received by the Company in the amount of \$0.6 million. As of March 31, 2019 and December 31, 2018, there were zero warrants exercisable for common shares outstanding.

NOTE 14. INCOME TAXES

The following table summarizes our effective tax rate and income tax expense for the three months ended March 31, 2019 and 2018 (dollars in millions):

	Three Months Ended March 31,	
	2019	2018
Effective tax rate	(1.0)%	(1.3)%
Income tax expense	\$ (0.4)	\$ (0.2)

NOTE 15. INVENTORY

Inventory, net of reserves, consisted of the following at March 31, 2019 and December 31, 2018 (in thousands):

	March 31, 2019	December 31, 2018
Raw materials	\$ 4,377	\$ 4,689
Finished goods	1,194	930
Total inventory	<u>\$ 5,571</u>	<u>\$ 5,619</u>

The inventory reserve was \$1.9 million and \$1.8 million at March 31, 2019 and December 31, 2018, respectively.

NOTE 16. ACCOUNTS RECEIVABLE

Accounts receivable, net of reserves for prompt pay discounts and doubtful accounts, was \$12.7 million and \$12.7 million at March 31, 2019 and December 31, 2018, respectively. The total reserves for both periods were immaterial.

NOTE 17. DISPOSITIONS

In June 2016, the company acquired certain rights to L-UDCA for \$0.5 million cash. At the same time the company established a related non-cash asset of \$25.5 million for IPR&D and a liability of \$25.0 million for contingent consideration (deferred financing) related net sales royalties and milestones. As a result of our quarterly valuation update process during 2016 and 2017, the contingent liability was decreased by \$2.3 million and \$5.7 million, respectively, and increased by \$1.0 million during 2018. The resulting balance of the L-UDCA contingent liability at December 31, 2018 was \$18.0 million.

During the first quarter of 2019, the company elected to discontinue development of the L-UDCA program, resulting in the write off of the intangible asset of \$25.5 million originally recorded in 2016, and the reversal of associated contingent consideration of \$18.0 million. This resulted in a net \$7.5 million non-cash charge to first quarter operations.

NOTE 18. SUBSEQUENT EVENT

On April 23, 2019, Retrophin, Inc. (the “Company”) entered into an office lease with an effective date of April 12, 2019 (the “Lease”) with Kilroy Realty, L.P. (the “Landlord”) for the lease of approximately 77,242 square feet of the building located at 3611 Valley Centre Drive, San Diego, California 92130 (the “Premises”). The Company expects to use the premises as its new principal corporate offices and plans to consolidate its corporate headquarters into the premises from the current location of multiple suites in adjacent buildings at 3721 and 3661 Valley Centre Drive, San Diego, California 92130. Under the terms of the Lease, the Company will have the one time right of first offer on the suites it currently occupies and a general right of first offer to lease additional space from the Landlord in the development. The commencement date of the Lease is expected to be October 1, 2020. The initial term of the Lease is 7 years, 7 months (the “Initial Term”), and the Landlord has granted the Company an option to extend the term of the Lease by a period of five years. The aggregate base rent due over the Initial Term under the terms of the Lease is approximately \$36.5 million.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in this Quarterly Report on Form 10-Q and the audited financial statements and notes thereto as of and for the year ended December 31, 2018 and the related Management’s Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our Annual Report on Form 10-K for the year ended December 31, 2018, filed with the Securities and Exchange Commission (SEC) on February 26, 2019. Past operating results are not necessarily indicative of results that may occur in future periods.

Forward-Looking Statements

The information in this discussion contains forward-looking statements and information within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which are subject to the “safe harbor” created by those sections. These forward-looking statements include, but are not limited to, statements concerning our strategy, future operations, future financial position, future revenues, projected costs, prospects and plans and objectives of management. The words “anticipates,” “believes,” “estimates,” “expects,” “intends,” “may,” “plans,” “projects,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make. These forward-looking statements involve risks and uncertainties that could cause our actual results to differ materially from those in the forward-looking statements, including, without limitation, the risks set forth in Part II, Item IA, “Risk Factors” in this Quarterly Report on Form 10-Q and in our other filings with the SEC. The forward-looking statements are applicable only as of the date on which they are made, and we do not assume any obligation to update any forward-looking statements.

Overview

We are a biopharmaceutical company headquartered in San Diego, California, focused on identifying, developing and delivering life-changing therapies to people living with rare diseases.

Our Product Candidates and Products That Have Been Approved

Program / Product	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Approved
Fosmetpantotenate (RE-024)	PKAN					
Sparsentan (RE-021)	FSGS					
	IgAN					
CNSA-001*	PKU					
NGLY1 Collaboration & Discovery Efforts	NGLY1 Deficiency					
						
						
						

* CNSA-001 is being developed in a strategic collaboration with Censa Pharmaceuticals.

We are developing the following pipeline products:

Fosmetpantotenate

We are developing fosmetpantotenate, a novel small molecule, as a potential treatment for pantothenate kinase-associated neurodegeneration (“PKAN”). PKAN is a genetic neurodegenerative disorder that is typically diagnosed in the first decade of life. Symptoms of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. PKAN is estimated to affect up to 5,000 patients worldwide. There are currently no viable treatment options for patients with PKAN. Fosmetpantotenate is a phosphopantothenate replacement therapy that aims to restore levels of this key substrate in PKAN patients. Certain international health regulators have approved the initiation of dosing fosmetpantotenate in PKAN patients under physician-initiated studies in accordance with local regulations in their respective countries.

In 2015 and 2016 we filed a U.S. Investigational New Drug application (“IND”), completed the Phase I clinical trials and obtained both orphan drug and fast track designation in the United States. Additionally, we received orphan drug designation in the European Union and reached an agreement with the FDA under the Special Protocol Assessment (SPA) process for a Phase 3 clinical trial for PKAN.

In the fourth quarter of 2018, the pivotal Phase 3 FORT Study of fosmetpantotenate in PKAN completed patient enrollment. The FORT Study is designed to be registration-enabling in the U.S. and Europe, and we expect top-line data to become available in the third quarter of 2019.

Sparsentan

Sparsentan is an investigational product candidate with a dual mechanism of action, a potent angiotensin receptor blocker (“ARB”) and selective endothelin receptor antagonist (“ERA”), with in vitro selectivity toward endothelin receptor type A. We have secured a license to sparsentan from Ligand Pharmaceuticals, Inc. and Bristol-Myers Squibb (who referred to it as DARA). Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in the following indications:

- Focal segmental glomerulosclerosis ("FSGS"), a leading cause of end-stage renal disease and nephrotic syndrome ("NS"). There are currently no FDA approved pharmacologic treatments for FSGS and off-label treatments are limited to ACE/ARBs, steroids, and immunosuppressant agents, which are effective in only a subset of patients. Every year approximately 5,400 patients are diagnosed with FSGS and we estimate that there are up to 40,000 FSGS patients in the United States with approximately half of them being candidates for sparsentan. In 2015 and 2016 we received orphan drug designation in the United States and European Union and generated positive data from our Phase 2 DUET study of sparsentan for the treatment of FSGS. In the second quarter of 2018, we announced the initiation of the Phase 3 DUPLEX Study of sparsentan in FSGS, and enrollment continues. This pivotal DUPLEX Study is designed to include an interim analysis of modified partial remission of proteinuria. We expect that successful achievement of this endpoint will serve as the basis for submission of a New Drug Application (NDA) for sparsentan for the treatment of FSGS under the Subpart H accelerated approval pathway in the United States and Conditional Marketing Authorization ("CMA") consideration in Europe. The confirmatory endpoint of the study will compare changes in slope of estimated glomerular filtration rate, or eGFR. Top-line data from the interim analysis are expected to become available in the second half of 2020.
- Immunoglobulin A nephropathy ("IgAN") is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of more than 100,000 people in the United States and greater numbers in Europe and Asia, IgAN is the most common primary glomerular disease. Most patients are diagnosed between the ages of 16 and 35, with up to 40% progressing to end stage renal disease within 15 years. There are currently no FDA approved treatments for IgAN. The current standard of care is renin-angiotensin-aldosterone system (RAAS) blockade with immunosuppression also being commonly used for patients with significant proteinuria or rapidly progressive glomerulonephritis. In the fourth quarter of 2018, we announced that the first patient had been dosed in the PROTECT Study, a global, pivotal Phase 3 clinical trial evaluating the long-term nephroprotective potential of sparsentan for the treatment of IgAN. The primary efficacy endpoint in the PROTECT Study is the change in proteinuria (urine protein-to-creatinine ratio) from baseline after 36 weeks of treatment. We expect that successful achievement of this endpoint will serve as the basis for submission of an NDA for sparsentan for the treatment of IgAN under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe. Secondary efficacy endpoints include change in eGFR from baseline to four weeks post-cessation of randomized treatment, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment. Top-line data from the primary endpoint are expected to become available in the first half of 2022.

CNSA-001

In December 2017, we entered into a Future Acquisition Right and joint development agreement with Censa Pharmaceuticals, Inc. ("Censa"), which became effective on January 4, 2018 upon the satisfaction of certain conditions. Pursuant to the agreement, we agreed to fund certain development activities of Censa's CNSA-001 program, in an aggregate amount expected to be approximately \$17 million through proof of concept, and have the right, but not the obligation, to acquire Censa (the "Option") on the terms and subject to the conditions set forth in a separate Agreement and Plan of Merger (the "Merger Agreement"). In exchange for the Option, we paid Censa \$10 million, and an additional \$5 million upon Censa's completion of a specified development milestone set forth in the Option Agreement, all of which will be distributed to Censa's equityholders.

Censa, a privately held biotechnology company focused on developing therapies for the orphan metabolic diseases, is developing CNSA-001 for the treatment of phenylketonuria ("PKU"). CNSA-001 is an orally bioavailable form of a natural precursor of tetrahydrobiopterin ("BH4") with the potential to provide improved phenylalanine ("Phe") reduction in patients with PKU when compared to BH4. Preclinical research has suggested CNSA-001 may provide improved bioavailability, plasma stability and tissue exposure, leading to higher intracellular BH4 levels and subsequent greater Phe reduction when compared to the current standard of care in PKU. In pre-clinical models, CNSA-001 has also shown an ability to cross the blood-brain barrier which, if supported by clinical data, may lead to broader utility in additional indications such as primary BH4 deficiency (PBD) and Segawa syndrome. CNSA-001 has completed both the single and multiple ascending dose studies. In October 2018, the first patient was dosed in the Phase 2 proof of concept study of CNSA-001 for PKU, and enrollment continues.

PKU is a rare, genetic metabolic condition in which the body cannot breakdown Phe due to a missing or defective phenylalanine hydroxylase ("PAH") enzyme. High Phe levels can lead to developmental and physical growth delay, executive function impairment, seizures, and microcephaly caused by toxic Phe accumulation in the brain. PKU is typically diagnosed at birth and has an estimated addressable population of 45,000 to 50,000.

If the Company exercises the Option, the Company will acquire Censa for an additional \$65 million, which may be reduced by up to \$2.6 million of development funding, paid as a combination of 20% in cash and 80% in shares of the Company's common stock, valued at a fixed price of \$21.40 per share; provided, however, that Censa may elect on behalf of its equity holders to receive the upfront consideration in 100% cash if the average price per share of the Company's common stock for the ten trading days ending on the date the Company provides a notice of interest to exercise the Option is less than \$19.26. In addition, if the Company exercises the Option and acquires Censa, the Company would be required to make further cash payments to Censa's equity holders of up to an aggregate of \$25 million if the CNSA-001 program achieves specified development and commercial milestones.

Under the terms of the agreement, we are providing funding for the development of CNSA-001 in PKU. Censa is responsible for the development program, which is being conducted under the oversight of a joint steering committee. As part of the agreement, we owe certain milestone payments upon achievement of specified milestones and we have the exclusive option to acquire Censa upon conclusion of a specified option period, pending clinical proof of concept of CNSA-001 in PKU.

Censa is currently conducting a Phase 2 proof-of-concept study evaluating CNSA-001 in patients with PKU and we expect top-line data to become available to us during the second quarter of 2019, with a decision made on the Option to acquire in the third quarter of 2019.

NGLY1 Deficiency Discovery Efforts

NGLY1 deficiency, is an extremely rare genetic disorder believed to be caused by a deficiency in an enzyme called N-glycanase-1, which is encoded by the gene NGLY1. The condition is characterized by symptoms such as developmental delays, seizures, complex hyperkinetic movement disorders, diminished reflexes and an inability to produce tears. There are no approved therapeutic options for NGLY1 deficiency, and current therapeutic strategies are limited to symptom management.

In September 2017, we entered into a three-way Cooperative Research and Development Agreement with the National Institutes of Health's National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency.

We sell the following three products:

Chenodal® (chenodiol tablets)

Chenodal is a synthetic oral form of chenodeoxycholic acid, a naturally occurring primary bile acid synthesized from cholesterol in the liver, indicated for the treatment of radiolucent stones in well-opacifying gallbladders in patients in whom selective surgery would be undertaken except for the presence of increased surgical risk due to systemic disease or age.

Chenodal administration is known to reduce biliary cholesterol and the dissolution of radiolucent gallstones through suppression of hepatic synthesis of cholesterol, cholic acid and deoxycholic acid in the bile pool. Chenodal was first approved by the the U.S. Food and Drug Administration ("FDA") in 1983 for the management of gallstones but its marketing was later discontinued due to lack of commercial success. In 2009, Nexgen Pharma Inc.'s Abbreviated New Drug Application ("ANDA") for Chenodal was approved by the FDA for the treatment of gallstones. Chenodal is manufactured under this ANDA. In 2010, Chenodal was granted orphan drug designation for the treatment of cerebrotendinous xanthomatosis ("CTX"), a rare autosomal recessive lipid storage disease. We acquired Chenodal in March 2014.

While Chenodal is not labeled for CTX, it has been used as the standard of care for over three decades. We are working to obtain FDA approval of Chenodal for the treatment of CTX. The prevalence of CTX is estimated in the literature to be as high as 1 in 70,000 in the overall population. Pathogenesis of CTX involves deficiency of the enzyme 27-hydroxylase (encoded by the gene CYP27A1), a rate-limiting enzyme in the synthesis of primary bile acids, including chenodeoxycholic acid ("CDCA"), from cholesterol. The disruption of primary bile acid synthesis in CTX leads to toxic accumulation of cholesterol and cholestanol in most tissues. Most patients present with intractable diarrhea, premature cataracts, tendon xanthomas, atherosclerosis, and cardiovascular disease in childhood and adolescence. Neurological manifestations of the disease, including dementia and cognitive and cerebellar deficiencies, emerge during late adolescence and adulthood. Oral administration of CDCA has been shown to normalize primary bile acid synthesis in patients with CTX.

Cholbam® (cholic acid capsules)

The FDA approved Cholbam (cholic acid capsules) in March 2015, the first FDA approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for adjunctive treatment of patients with peroxisomal disorders (including Zellweger spectrum disorders). The effectiveness of Cholbam has been demonstrated in clinical trials for bile acid synthesis disorders and the adjunctive treatment of peroxisomal disorders. An estimated 200 to 300 patients are current candidates for therapy.

Kolbam, the branded name of Cholbam in Europe, is indicated in Europe for the treatment of certain inborn errors of primary bile acid synthesis, encompassing select single enzyme defects, in infants from one month of age for continuous lifelong treatment through adulthood.

Thiola® (tiopronin)

Thiola is approved by the FDA for the treatment of cystinuria, a rare genetic cystine transport disorder that causes high cystine levels in the urine and the formation of recurring kidney stones. The resulting long-term damage can cause loss of kidney function in addition to substantial pain and loss of productivity associated with renal colic and stone passage. The prevalence of cystinuria in the United States is estimated to be 10,000 to 12,000, indicating that there may be as many as 4,000 to 5,000 affected individuals with cystinuria in the United States that would be candidates for Thiola. An NDA has been filed for a new, more patient-friendly, formulation of Thiola and the FDA has assigned a Prescription Drug User Fee Act ("PDUFA") target action date of June 30, 2019.

Pipeline portfolio changes:

During the first quarter of 2019, the company elected to discontinue development of the L-UDCA program, resulting in removing the intangible asset of \$25.5 million which was originally recorded in 2016 and the related \$18.0 million in contingent liability. This resulted in a net \$7.5 million non-cash charge to first quarter operations.

In June 2016, the company acquired certain rights to L-UDCA for \$0.5 million cash. At the same time the company established a related non-cash asset of \$25.5 million and liability of \$25.0 million for IPR&D and contingent consideration (deferred financing) related net sales royalties and milestones. As a result of our quarterly valuation update process during 2016 and 2017, the contingent liability was decreased by \$2.3 million and \$5.7 million, respectively, and increased by \$1.0 million during 2018. The resulting balance of the L-UDCA contingent liability at December 31, 2018 was \$18.0 million.

Results of Operations

Results of operations for the three months ended March 31, 2019 compared to the three months ended March 31, 2018.

Net Product Sales:

The following table provides information regarding net product sales (*in thousands*):

	Three Months Ended March 31,		
	2019	2018	Change
Net product revenues by product:			
Bile acid products	\$ 18,390	\$ 18,508	\$ (118)
Thiola	21,180	19,924	1,256
Total net product revenues	<u>\$ 39,570</u>	<u>\$ 38,432</u>	<u>\$ 1,138</u>

The sales increase for the three months ended March 31, 2019 compared to the three months ended March 31, 2018 was due to increased patient counts, as well as the normal fluctuations in timing of new patient starts and prescription refills.

Operating Expenses:

The following table provides information regarding operating expenses (*in thousands*):

	Three Months Ended March 31,		
	2019	2018	Change
Cost of goods sold	\$ 1,017	\$ 1,613	\$ (596)
Research and development	33,443	24,636	8,807
Selling, general and administrative	32,669	26,468	6,201
Change in fair value of contingent consideration	3,169	3,627	(458)
Impairment of L-UDCA IPR&D intangible asset	25,500	—	25,500
Write off of L-UDCA contingent consideration	(18,000)	—	(18,000)
	<u>\$ 77,798</u>	<u>\$ 56,344</u>	<u>\$ 21,454</u>

Research and development expenses

We make significant investments in research and development in support of our development programs. Research and development costs are expensed as incurred and include salaries and bonuses, benefits, non-cash share based compensation, license fees, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials, and associated overhead expenses and facility costs.

For the three months ended March 31, 2019 as compared to the three months ended March 31, 2018, the Company increased its research and development expenses by \$8.8 million, which is due to increased clinical trial expenses related to our three on-going Phase 3 studies, fosmetpantotenate in PKAN and sparsentan in FSGS and IgAN.

Selling, general and administrative expenses

Selling, general and administrative expenses include salaries and bonuses, benefits, non-cash share based compensation, professional fees, rent, depreciation and amortization, travel, insurance, business development, sales and marketing programs, and other operating expenses.

For the three months ended March 31, 2019 as compared to the three months ended March 31, 2018, the Company increased its selling, general and administrative expenses by \$6.2 million due to compensation expense, primarily as a result of a larger employee base, and increased legal expenses.

Change in the valuation of contingent consideration

For the three months ended March 31, 2019 as compared to the three months ended March 31, 2018, the change in fair value of contingent consideration is due to timing of payments for Chenodal and Cholbam.

The following table summarizes the Company's change in valuation of contingent consideration (*in thousands*):

	Three Months Ended March 31,		
	2019	2018	Change
Chenodal	\$ 1,539	\$ 801	\$ 738
Cholbam	1,630	1,826	(196)
L-UDCA	—	1,000	(1,000)
Change in fair value of contingent consideration	<u>\$ 3,169</u>	<u>\$ 3,627</u>	<u>\$ (458)</u>

Write off of L-UDCA Contingent Consideration and impairment of L-UDCA IPR&D intangible assets

In June 2016, the company acquired certain rights to L-UDCA for \$0.5 million cash. At the same time the company established a related non-cash asset of \$25.5 million and liability of \$25.0 million for IPR&D and contingent consideration (deferred financing) related net sales royalties and milestones. As a result of our quarterly valuation update process during 2016 and 2017, the contingent liability was decreased by \$2.3 million and \$5.7 million, respectively, and increased by \$1.0 million during 2018. The resulting balance of the L-UDCA contingent liability at December 31, 2018 was \$18.0 million.

During the first quarter of 2019, the company elected to discontinue development of the L-UDCA program, resulting in removing the intangible asset of \$25.5 million which was originally recorded in 2016, and the reversal of associated contingent consideration of \$18.0 million. This resulted in a net \$7.5 million non-cash charge to first quarter operations.

Other Income/(Expenses):

The following table provides information regarding other income (expenses), net (*in thousands*):

	Three Months Ended March 31,		
	2019	2018	Change
Other income (expense), net	\$ (302)	\$ 121	\$ (423)
Interest income	2,819	797	2,022
Interest expense	(4,865)	(1,155)	(3,710)
	<u>\$ (2,348)</u>	<u>\$ (237)</u>	<u>\$ (2,111)</u>

The change in the Company's other income (expenses) for the three months ended March 31, 2019 as compared to the three months ended March 31, 2018 of \$2.1 million is primarily due to the additional interest from the Company's 2.50% Convertible Senior Notes due in 2025 ("2025 Notes") issued in September 2018.

Income Tax Benefit:

For the three months ended March 31, 2019, we recognized an income tax expense of \$0.4 million, representing an effective tax rate of 1.0%. Under GAAP, quarterly effective tax rates may vary significantly depending on the actual operating results in the various tax jurisdictions, and significant transactions, as well as changes in the valuation allowance related to the expected recovery of deferred tax assets.

For the three months ended March 31, 2019, as compared to the same period in 2018, the change in the tax expense and decrease in the effective income tax rate was primarily attributable to the increase in state tax expense in 2019.

The difference between the expected statutory federal tax benefit of 21% and the effective tax expense for three months ended March 31, 2019, was primarily attributable to the valuation allowance against our deferred tax assets.

At March 31, 2019, we had no unrecognized tax benefits. We did not recognize any interest or penalties related to unrecognized tax benefits during the three months ended March 31, 2019.

Liquidity and Capital Resources

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations for at least the next 12 months. Management believes that our operating results will vary from quarter to quarter and year to year depending upon various factors including revenues, general and administrative expenses, and research and development expenses.

The Company had the following financial performance at March 31, 2019 and December 31, 2018 (*in thousands*):

	March 31, 2019	December 31, 2018
Cash & Cash Equivalents	\$ 69,838	\$ 102,873
Marketable securities	377,808	368,668
Accumulated Deficit	(310,994)	(270,017)
Stockholders' Equity	285,711	318,253
Net Working Capital*	\$ 371,438	\$ 391,057
Net Working Capital Ratio**	4.57	4.74

* Current assets less current liabilities.

**Current assets divided by current liabilities.

Convertible Notes Payable

Convertible Senior Notes Due 2025

On September 10, 2018, the Company completed its registered underwritten public offering of \$276 million aggregate principal amount of the 2025 Notes, and entered into a base indenture and supplemental indenture agreement ("2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025 ("Maturity Date"), unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year, the first payment was made on March 15, 2019.

The composition of the Company's 2025 Notes are as follows (*in thousands*):

	March 31, 2019	December 31, 2018
2.50% convertible senior notes due 2025	\$ 276,000	\$ 276,000
Unamortized debt discount	(72,681)	(74,836)
Unamortized debt issuance costs	(5,849)	(6,073)
Total 2025 Notes, net of unamortized debt discount and debt issuance costs	<u>\$ 197,470</u>	<u>\$ 195,091</u>

The net proceeds from the issuance of the 2025 Notes were approximately \$267.2 million, after deducting commissions and the offering expenses payable by the Company. A portion of the net proceeds from the 2025 Notes were used by the Company to repurchase \$23.4 million aggregate principal amount of its then-outstanding 4.50% Senior Convertible Notes due in 2019 ("2019 Notes") in privately-negotiated transactions for approximately \$40.2 million in cash.

Holders may convert their 2025 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on December 31, 2018 (and only during such calendar quarter), if the last reported sale price per share of the Company's common stock for each of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period (such 10 consecutive trading day period, the "measurement period") if the trading price per \$1,000 principal amount of 2025 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of the Company's common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain corporate events or distributions on the Company's common stock; (4) if the Company calls the 2025 Notes for redemption; and (5) at any time from, and including, May 15, 2025 until the close of business on the scheduled trading day immediately before the Maturity Date. The Company will settle conversions by paying or delivering, as applicable, cash, shares of the Company's common stock, or a combination of cash and shares of the Company's common stock, at the Company's election, based on the applicable conversion rate.

The initial conversion rate for the 2025 Notes is 25.7739 shares of the Company's common stock per \$1,000 principal amount of 2025 Notes, which represents an initial conversion price of approximately \$38.80 per share. If a "make-whole fundamental change" (as defined in the 2025 Indenture) occurs, then the Company will in certain circumstances increase the conversion rate for a specified period of time.

The 2025 Notes will be redeemable, in whole or in part, at the Company's option at any time, and from time to time, on or after September 15, 2022 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the Maturity Date, at a cash redemption price equal to the principal amount of the 2025 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date but only if the last reported sale price per share of the Company's common stock exceeds 130% of the conversion price on each of at least 20 trading days during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related redemption notice. If a fundamental change (as defined in the 2025 Indenture) occurs, then, subject to certain exceptions, holders may require the Company to repurchase their 2025 Notes at a cash repurchase price equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date.

In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2025 Notes will be paid pursuant to the terms of the 2025 Indenture. In

the event that all of the 2025 Notes are converted, the Company would be required to repay the \$276.0 million in principal value and any conversion premium in any combination of cash and shares of its common stock (at the Company's option).

Convertible Senior Notes Due 2019

On May 29, 2014, the Company entered into a Note Purchase Agreement relating to a private placement by the Company of \$46.0 million aggregate principal of 2019 Notes which are convertible into shares of the Company's common stock at an initial conversion price of \$17.41 per share. The conversion price is subject to customary anti-dilution protection. The 2019 Notes bear interest at a rate of 4.5% per annum, payable semiannually in arrears on May 15 and November 15 of each year. The 2019 Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with their terms, and there are no contractual payments due prior to that date.

In September 2018, the Company used part of the net proceeds from the issuance of the 2025 Notes noted above to repurchase \$23.4 million aggregate principal value of the 2019 Notes in privately-negotiated transactions for approximately \$40.2 million in cash. The partial repurchase of the 2019 Notes resulted in a \$17.0 million loss on early extinguishment of debt in September 2018. At March 31, 2019, approximately \$22.6 million of principal remained outstanding on the 2019 Notes.

The composition of the Company's 2019 Notes are as follows (*in thousands*):

	March 31, 2019	December 31, 2018
4.50% senior convertible notes due 2019	\$ 22,590	\$ 22,590
Unamortized debt discount	(50)	(125)
Unamortized debt issuance costs	(3)	(8)
Total 2019 Notes, net of debt discount and debt issuance costs	<u>\$ 22,537</u>	<u>\$ 22,457</u>

Cash Flows from Operating Activities

Cash used in operating activities was \$20.5 million for the three months ended March 31, 2019 compared to cash used of \$14.7 million for the three months ended March 31, 2018. The increase in cash used is attributable to net changes in operating assets and liabilities and increased cash used in payments related to change in fair value of contingent consideration.

Cash Flows from Investing Activities

Cash used by investing activities for the three months ended March 31, 2019 was \$11.4 million, compared to cash used of \$20.9 million for the three months ended March 31, 2018. The change was primarily due to the \$10 million option payment to CENSA pursuant to the Future Acquisition Right and Joint Development Agreement recorded as cash paid for investments - equity.

Cash Flows from Financing Activities

Cash used by financing activities for the three months ended March 31, 2019 was \$1.1 million compared to cash used of \$2.7 million for the three months ended March 31, 2018. The change was due to lower contingent consideration payments in 2019, offset by lower proceeds from the exercise of stock options and warrants. In the three months ended March 31, 2018, the Company paid a net sales milestone payment for Cholbam.

Funding Requirements

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations for at least the next 12 months. This belief is based on many factors, some of which are beyond our control. Factors that may affect financing requirements include, but are not limited to:

- revenue growth of our marketed products;
- the rate of progress and cost of our clinical trials, preclinical studies and other discovery and research and development activities;
- the timing of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- debt service obligations on the 2019 Notes, which mature on May 30, 2019;
- debt service obligations on the 2025 Notes;
- our ability to manufacture sufficient quantities of our products to meet expected demand;
- the costs of preparing, filing, prosecuting, maintaining and enforcing any patent claims and other intellectual property rights, litigation costs and the results of litigation;
- our ability to enter into collaboration, licensing or distribution arrangements and the terms and timing of these arrangements;
- the potential need to expand our business, resulting in additional payroll and other overhead expenses;
- the potential in-licensing of other products or technologies; and

- the emergence of competing technologies or other adverse market or technological developments.

Future capital requirements will also depend on the extent to which we acquire or invest in additional complementary businesses, products and technologies.

Other Matters

Adoption of New Accounting Standards

See Note 2 to our unaudited Condensed Consolidated Financial Statements in this report for a discussion of adoption of new accounting standards.

Recently Issued Accounting Pronouncements

See Note 2 to our unaudited Condensed Consolidated Financial Statements in this report for a discussion of recently issued accounting pronouncements.

Off Balance Sheet Arrangements

In December 2017, the Company entered into a Future Acquisition Right of Joint Development Agreement with Censa, which became effective in January 2018. The Company determined that Censa is a variable interest entity ("VIE") and concluded that the Company is not the primary beneficiary of the VIE. As such, the Company did not consolidate Censa's results into its consolidated financial statements. The Company will continue to monitor facts and circumstances for changes that could potentially result in the Company becoming the primary beneficiary. See Note 5 to the Unaudited Condensed Consolidated Financial Statements.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

We invest our excess cash and marketable securities primarily in United States government backed securities, asset-backed securities, and debt instruments of financial institutions and corporations with investment-grade credit ratings. These instruments have various short and long-term maturities, not exceeding two years. We do not utilize derivative financial instruments, derivative commodity instruments, or other market risk sensitive instruments, positions or transactions. Accordingly, we believe that, while the instruments held are subject to changes in the financial standing of the issuer of such securities, we are not subject to any material risks arising from changes in interest rates, foreign currency exchange rates, commodity prices, equity prices or other market changes that affect market risk sensitive investments. A hypothetical 1% adverse move in interest rates along the entire interest rate yield curve would decrease our available for sale marketable securities by approximately \$1.7 million if the Company were to sell the securities.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our reports required by the Exchange Act of 1934, as amended, is recorded, processed, summarized and reported within the timelines specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and in reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

As required by SEC Rule 13a-15(b), we carried out an evaluation, under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the quarter covered by this report. Based on the foregoing, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

An evaluation was also performed under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of any change to our internal control over financial reporting that occurred during the quarter covered by this report and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. Our evaluation did not identify significant changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934) that occurred during the quarter ended March 31, 2019, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II - OTHER INFORMATION

Item 1. Legal Proceedings

The information required by this Item is incorporated herein by reference to the Notes to the Unaudited Condensed Consolidated Financial Statements--Note 12 Commitments and Contingencies: Legal Proceedings in Part I, Item 1, of this Quarterly Report on Form 10-Q.

Item 1A. Risk Factors

The following risk factors do not reflect any material changes to the risk factors set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018, other than the revisions to the risk factors set forth below with an asterisk (*) next to the title. The following information sets forth risk factors that could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. If any of the following risks actually occur, our business, operating results, prospects or financial condition could be harmed. Additional risks not presently known to us, or that we currently deem immaterial, may also affect our business operations.

Risks Related to the Development of our Product Candidates

Our clinical trials may fail to demonstrate the safety and efficacy of our product candidates which could prevent or significantly delay their regulatory approval.

Before obtaining regulatory approval for the sale of any of our product candidates, we must subject these product candidates to extensive preclinical and clinical testing to demonstrate their safety and efficacy for humans. Clinical trials are expensive, time-consuming and may take years to complete.

We may experience numerous unforeseen events during, or as a result of, preclinical or nonclinical testing and the clinical trial process that could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, including:

- our preclinical or nonclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;
- regulators may require us to conduct studies of the long-term effects associated with the use of our product candidates;
- regulators or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- the FDA or any non-United States regulatory authority may impose conditions on us regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;
- our third-party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- we might have to suspend or terminate one or more of our clinical trials if we, regulators or institutional review boards determine that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and
- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

These risks and uncertainties impact all of our clinical programs. We will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA, and in the case of foreign commercialization, to the applicable foreign regulatory authorities, in well-designed and conducted clinical trials, that our product candidates are safe and effective and otherwise meet the appropriate standards required for approval for a particular indication.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products or product candidates.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;
- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; and
- have the product removed from the market after obtaining marketing approval.

Our product candidates are intended to treat PKAN, FSGS and IgAN, each of which is a rare disease. Given that these development candidates are still undergoing required testing, we may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible patients willing and able to participate in the clinical trials required by the FDA or foreign regulatory agencies. In addition, as other companies and researchers may be concurrently developing therapies for the same or similar indications that we are focused on, we could face competition for a limited number of patients, investigators and clinical trial sites willing to participate in clinical trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results. For example, there can be no assurance that the favorable responses we have seen with the physician-initiated treatment of fosmetpantotenate in PKAN patients outside the United States will translate to positive data in the Phase 3 clinical trial of fosmetpantotenate or that the positive results from the DUET study of sparsentan in FSGS will be repeated in the Phase 3 clinical trial. Similarly, there can be no assurance that our clinical experience with sparsentan in FSGS will translate to favorable data in IgAN, which patient population has

not previously been treated with sparsentan prior to the Phase 3 trial currently being conducted. We cannot assure that any current or future clinical trials of fosmetpantotenate, sparsentan and/or CNSA-001 will ultimately be successful.

Before obtaining regulatory approval to conduct clinical trials of our product candidates, we must conduct extensive preclinical tests to demonstrate the safety of our product candidates in animals. Preclinical testing is expensive, difficult to design and implement, and can take many years to complete. In addition, during the clinical development process, additional nonclinical toxicology studies are routinely conducted concurrently with the clinical development of a product candidate. If any of our product candidates show unexpected findings in concurrent toxicology studies, we could experience potentially significant delays in, or be required to abandon, development of that product candidate. A failure of one or more of our nonclinical studies can occur at any stage of testing.

Communications and/or feedback from the FDA related to our current or planned future clinical trials does not guarantee any particular outcome from regulatory review for such clinical trials.

Communications and/or feedback from the FDA related to our current or future clinical trials does not guarantee any particular outcome from regulatory review for such clinical trials. For example, although we have obtained a Special Protocol Assessment ("SPA") agreement from the FDA for the Phase 3 clinical trial of fosmetpantotenate for the treatment of PKAN, this agreement does not guarantee any particular outcome from regulatory review. The SPA is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of an NDA. However, a SPA is not a guarantee of an approval of a product candidate or any permissible claims about the product candidate. In particular, a SPA agreement is not binding on the FDA if previously unrecognized public health concerns arise during the performance of the clinical trial, if other new scientific concerns regarding product candidate safety or efficacy arise or if the sponsoring company fails to comply with the agreed upon clinical trial protocols. Moreover, a SPA does not address all of the variables and details that may go into planning for or conducting a clinical trial, and changes in the protocol for a clinical trial can invalidate a SPA or require that the FDA agree in writing to the modified protocol. In addition, while a SPA addresses the requirements for submission of an NDA, the results of the related clinical trial may not support FDA approval. There can be no assurance that the Phase 3 clinical trial for fosmetpantotenate will demonstrate that fosmetpantotenate is safe and effective for treating PKAN or that the data will support an application for approval by the FDA. In the third quarter of 2019, we plan to un-blind the data from our ongoing pivotal Phase 3 trial of fosmetpantotenate. If the results of this trial are not positive, or are not viewed sufficiently favorably, the market price of our common stock could decline significantly. In addition, following the initial 24 week study period, patients in this trial are eligible to participate in an open label extension. Even if the initial un-blinded results from the trial are viewed positively, it is possible that negative consequences of the treatment could be observed in the future, which could adversely impact the regulatory and commercial prospects of fosmetpantotenate and our business may suffer.

In addition, in 2018, we initiated the following Phase 3 clinical trials of sparsentan: 1) a single Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of FSGS (the "DUPLEX Study"), and 2) a single Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of IgAN (the "PROTECT Study"). We are conducting the DUPLEX Study and the PROTECT Study under the Subpart H pathway for potential accelerated approval in the US, and in the EU we plan to pursue potential Conditional Marketing Authorization, in both jurisdictions based on change in proteinuria. Recognition of change in proteinuria as a surrogate endpoint in kidney disease is a relatively new regulatory development, and, as the field continues to evolve, new learnings may impact regulatory viewpoints. We expect that the FDA's and EMA's determination as to whether the sufficiency of the data supports an accelerated approval in either jurisdiction will be made during the application review process. There can be no assurance that even if we achieve statistical significance on the interim or primary endpoints for the DUPLEX Study and/or the PROTECT Study, as applicable, that the FDA or EMA will deem that sufficient to grant accelerated approval or Conditional Marketing Authorization.

Although we received feedback from the FDA at an End of Phase 2 meeting for the sparsentan FSGS program during which the FDA communicated that it was open to accepting a substantial treatment effect on proteinuria in the DUPLEX Study as a basis for accelerated approval pursuant to Subpart H of the FDA regulations and although we subsequently gained alignment that our statistical modeling supported initiating a Phase 3 trial that proceeds on the Subpart H pathway, there can be no guarantee that the data generated from the study will be sufficient to serve as the basis for an NDA filing, including an NDA under Subpart H for accelerated approval. In addition, our statistical modeling that supports proceeding with the Duplex Study on the Subpart H pathway is based on data from other FSGS studies. To the extent that the model population is not representative of the Duplex Study population, the FDA may not agree that the new results continue to support a Subpart H pathway. Furthermore, even if sparsentan is granted accelerated approval for FSGS, there can be no assurance that the post-marketing confirmatory data will support full approval of sparsentan as a treatment for FSGS.

Also, although we have reached agreement with the FDA regarding the initiation of the PROTECT Study and the trial began in December 2018, we continue to have regulatory interactions regarding certain details of the study. There can be no assurance that the study will proceed as planned and there can be no guarantee that the data generated from the study will be sufficient to serve as the basis for an NDA filing, including an NDA under Subpart H for accelerated approval or support Conditional Marketing Authorization in the EU. Furthermore, even if sparsentan is granted accelerated approval for IgAN, there can be no assurance that the post-marketing confirmatory data will support full approval of sparsentan as a treatment for IgAN.

In addition, because both the DUPLEX Study and PROTECT Study are evaluating the same compound for the treatment of chronic kidney diseases and utilizing similar endpoints, the risk of success or failure for the two studies may, depending on the outcomes of the studies, end up being correlated.

Even if we receive regulatory approval for any product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense.

Any regulatory approvals that we receive for any product candidates may be subject to significant limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

In addition, if the FDA or a comparable foreign regulatory authority approves any product candidates, those products will be subject to extensive and ongoing regulatory requirements, including for the manufacturing, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export, recordkeeping, conduct of potential post-marketing studies and post-market submission requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with current good manufacturing practices and good clinical practices, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, undesirable side effects caused by the product, problems encountered by our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, either before or after product approval, may result in, among other things:

- restrictions on the marketing, manufacturing, or distribution of the product;
- requirements to include additional warnings on the label;
- requirements to create or enhance a medication guide outlining the risks to patients;
- withdrawal of the product from the market;
- voluntary or mandatory product recalls;
- requirements to change the way the product is administered or for us to conduct additional clinical trials;
- fines, warning or untitled letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties; and
- harm to our reputation.

For example, we have certain post-marketing requirements and commitments associated with Cholbam. Further, we face risks relating to the post marketing obligations and commercial acceptance of Cholbam, which was approved by the FDA on March 17, 2015. If the regulatory approval for Chenodal, Cholbam and/or Thiola are withdrawn for any reason, it would have a material adverse impact on our sales and profitability.

The independent clinical investigators and contract research organizations that we rely upon to conduct our clinical trials may not be diligent, careful or timely, and may make mistakes, in the conduct of our trials.

We depend on independent clinical investigators and contract research organizations (“CROs”) to conduct our clinical trials under agreements with us. The CROs play a significant role in the conduct of our clinical trials. Failure of the CROs to meet their obligations could adversely affect clinical development of our product candidates. The independent clinical investigators are not our employees and we cannot control the timing or amount of resources they devote to our studies. If their performance is substandard, it could delay or prevent approval of our FDA applications. Moreover, these independent investigators and CROs may also have relationships with other commercial entities, some of which may compete with us. If independent investigators and CROs allocate their resources to assist our competitors at our expense, it could harm our competitive position.

We have limited control over the development activities of Censa’s CNSA-001 program and our investment in Censa’s CNSA-001 program may be adversely affected.

We have entered into a joint development agreement with Censa to evaluate CNSA-001 for the treatment of PKU. Under this agreement, we have agreed to fund certain development activities of Censa’s CNSA-001 program, in an aggregate amount expected to be approximately \$19.7 million through proof of concept. However, we have limited control over the development activities of Censa’s CNSA-001 program and face the risk that the development program for CNSA-001 will not be successful, that Censa does not conduct the development activities in a timely manner, or that the development program for CNSA-001 may cost more than expected to reach proof of concept. If any of these issues arise, our investment in Censa’s CNSA-001 program may be adversely affected and our business may suffer.

Risks Related to the Commercialization of Our Products

The commercial success of Chenodal, Cholbam and Thiola depends on them being considered to be effective drugs with advantages over other therapies.

The commercial success of our products Chenodal, Cholbam and Thiola depends on them being considered to be effective drugs with advantages over other therapies. A number of factors, as discussed in greater detail below, may adversely impact the degree of acceptance of these products, including their efficacy, safety, price and benefits over competing therapies, as well as the coverage and reimbursement policies of third-party payers, such as government and private insurance plans.

If unexpected adverse events are reported in connection with the use of any of these products, physician and patient acceptance of the product could deteriorate and the commercial success of such product could be adversely affected. We are required to report to the FDA events associated with our products relating to death or injury. Adverse events could result in additional regulatory controls, such as a requirement for costly post-approval clinical studies or revisions to our approved labeling which could limit the indications or patient population for a product or could even lead to the withdrawal of a product from the market.

***A number of recent regulatory and legislative initiatives have been introduced to encourage generic competition for pharmaceutical products, and if a generic version of any of our products enters the market, sales of that product would be negatively impacted.**

Chenodal and Thiola are subject to immediate competition from compounded and generic entrants, as the ANDA and NDA for these drug products have no remaining patent or nonpatent exclusivity. There have been a number of recent regulatory and legislative initiatives designed to encourage generic competition for pharmaceutical products, including expedited review procedures for generic manufacturers and incentives designed to spur generic competition of branded drugs. In October 2018, we were named as a defendant in a lawsuit brought by a purported generic company seeking to acquire samples of Thiola in order to conduct bioequivalence studies.

Also, the FDA and the U.S. Federal Trade Commission have been focused on brand companies' denial of drug supply to potential generic competitors for testing, and the U.S. Congress has been considering a legislatively defined private right of action under which generic companies could bring suit against companies who refuse access to product for bioequivalence testing. We cannot currently predict the specific outcome or impact on our business of such regulatory actions or legislation. It is our policy to evaluate requests for samples of our branded products, and to provide samples in response to bona fide requests from certain qualified third parties, including generic manufacturers, subject to specified conditions.

Under the Hatch-Waxman Amendments of the Federal Food, Drug, and Cosmetic Act (the "FDC Act"), a pharmaceutical manufacturer may file an ANDA, seeking approval of a generic copy of an approved innovator product or an NDA under Section 505(b)(2) that relies on the FDA's prior findings of safety and effectiveness in approving the innovator product. A Section 505(b)(2) NDA may be for a new or improved version of the original innovator product. The Hatch-Waxman Amendments also provide for certain periods of regulatory exclusivity, which preclude FDA approval (or in some circumstances, FDA acceptance) of an ANDA or Section 505(b)(2) NDA. In addition, the FDC Act provides, subject to certain exceptions, a period during which an FDA-approved drug may be afforded orphan drug exclusivity. In addition to the benefits of regulatory exclusivity, an innovator NDA holder may have patents claiming the active ingredient, product formulation or an approved use of the drug, which would be listed with the product in the FDA publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," known as the "Orange Book." If there are patents listed in the Orange Book, a generic or Section 505(b)(2) applicant that seeks to market its product before expiration of the patents must include in the ANDA what is known as a "Paragraph IV certification," challenging the validity or enforceability of, or claiming non-infringement of, the listed patent or patents. Notice of the certification must be given to the innovator, too, and if within 45 days of receiving notice the innovator sues to enforce its patents, approval of the ANDA is stayed for 30 months, or as lengthened or shortened by the court.

If a generic version of Chenodal, Thiola or any of our products is approved, sales of that product would be negatively impacted, which would have a material adverse impact on our sales and profitability.

***Changes in reimbursement practices of third-party payers could affect the demand for our products and the prices at which they are sold.**

The business and financial condition of healthcare-related businesses will continue to be affected by efforts of governments and third-party payers to contain or reduce the cost of healthcare through various means. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval for fosmetpantotenate, sparsentan and CNSA-001, or any other product candidate that we develop, restrict or regulate post-approval activities and affect our ability to profitably sell fosmetpantotenate, sparsentan, and CNSA-001 or any other product candidate for which we obtain marketing approval.

Our products are sold to patients whose healthcare costs are met by third-party payers, such as government programs, private insurance plans and managed-care programs. These third-party payers are increasingly attempting to contain healthcare costs by limiting both coverage and the level of reimbursement for medical products and services. Levels of reimbursement, if any, may be decreased in the future, and future healthcare reform legislation, regulations or changes to reimbursement policies of third party payers may otherwise adversely affect the demand for and price levels of our products, which could have a material adverse effect on our sales and profitability.

Economic, social, and congressional pressure may result in individuals and government entities increasingly seeking to achieve cost savings through mechanisms that limit coverage or payment for our products. For example, state Medicaid programs are increasingly requesting

manufacturers to pay supplemental rebates and requiring prior authorization for use of drugs. Managed care organizations continue to seek price discounts and, in some cases, to impose restrictions on the coverage of particular drugs. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding constraint on prices and reimbursement for our products.

We are dependent on third parties to manufacture and distribute our pharmaceutical products who may not fulfill their obligations.

We have no manufacturing capabilities and rely on third party manufacturers who are sole source suppliers for manufacturing of Chenodal, Cholbam and Thiola. The facilities used by our third party manufacturers must be approved by the FDA, or in the case of Kolbam in the European Union, the European Medicines Agency. Our dependence on third parties for the manufacture of our products may harm our profit margin on the sale of products and our ability to deliver products on a timely and competitive basis. If our third party manufacturers are unable to manufacture to specifications or in compliance with applicable regulatory requirements, our ability to commercialize our products will be adversely impacted and could affect our ability to gain market acceptance for our products and negatively impact our revenues.

We currently have no in-house distribution channels for Chenodal, Cholbam or Thiola and we are dependent on a third-party distributor, Dohmen Life Sciences Services, an Eversana Company, to distribute such products. We rely on this distributor for all of our proceeds from sales of Chenodal, Cholbam and Thiola in the United States. The outsourcing of our distribution function is complex, and we may experience difficulties that could reduce, delay or stop shipments of such products. If we encounter such distribution problems, and we are unable to quickly enter into a similar agreement with another distributor on substantially similar terms, distribution of Chenodal, Cholbam and/or Thiola could become disrupted, resulting in lost revenues, provider dissatisfaction, and/or patient dissatisfaction.

Governments outside the United States tend to impose strict price controls and reimbursement approval policies, which may adversely affect our prospects for generating revenue.

In some countries, particularly European Union countries, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time (6 to 12 months or longer) after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our prospects for generating revenue outside of the United States, if any, could be adversely affected and our business may suffer.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate product revenue outside of the United States.

We may not be able to rely on orphan drug exclusivity for Cholbam/Kolbam or any of our products.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. We have obtained orphan designation for Cholbam/Kolbam in the United States and the European Union. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in Europe. Even though we have been awarded orphan drug exclusivity for Cholbam in the United States, we may not be able to maintain it. For example, if a competitive product that contains the same active moiety and treats the same disease as our product is shown to be clinically superior to our product, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose orphan drug exclusivity. Similarly, if a competitive product that contains the same active moiety and treats the same disease as our product candidate is approved for orphan drug exclusivity before our product candidate, we may not be able to obtain approval for our product candidate until the expiration of the competitive product's orphan drug exclusivity unless our product candidate is shown to be clinically superior to the competitive product.

Risks Related to our Products and Product Candidates

***Our products may not achieve or maintain expected levels of market acceptance or commercial success.**

The success of our products is dependent upon achieving and maintaining market acceptance. Commercializing products is time consuming, expensive and unpredictable. There can be no assurance that we will be able to, either by ourselves or in collaboration with our partners or through our licensees, successfully commercialize new products or current products or gain market acceptance for such products. New product candidates that appear promising in development may fail to reach the market or may have only limited or no commercial success.

Further, the discovery of significant problems with a product similar to one of our products that implicate (or are perceived to implicate) an entire class of products could have an adverse effect on sales of the affected products. Accordingly, new data about our products, or products similar to our products, could negatively impact demand for our products due to real or perceived side effects or uncertainty regarding efficacy and, in some cases, could result in product withdrawal.

Our current products and any products that we bring to the market, including fosmetopantotenate, sparsentan and CNSA-001, if they receive marketing approval, may not gain market acceptance by physicians, patients, third-party payers, and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The

degree of market acceptance of our current products and product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage and reimbursement.

Even if a potential or current product displays a favorable efficacy and safety profile in preclinical and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate patients, the medical community, and third-party payers on the benefits of our product may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional marketing technologies employed by our competitors.

If the market opportunities for our products and product candidates are smaller than we believe they are, our revenues may be adversely affected and our business may suffer.

Certain of the diseases that our current and future product candidates are being developed to address, such as PKAN, PKU, FSGS and IgAN, are relatively rare. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, may not be accurate.

Currently, most reported estimates of the prevalence of PKAN and FSGS are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. As new studies are performed the estimated prevalence of these diseases may change. There can be no assurance that the prevalence of PKAN and FSGS in the study populations accurately reflect the prevalence of these diseases in the broader world population. If our estimates of the prevalence of PKAN, PKU, FSGS, or IgAN or of the number of patients who may benefit from treatment with fosmetpantotate, sparsentan, and CNSA-001 prove to be incorrect, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.

Undesirable side effects caused by our product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product candidates and generating revenues from their sale.

In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may require the addition of restrictive labeling statements;
- regulatory authorities may withdraw their approval of the product; and
- we may be required to change the way the product is administered or conduct additional clinical trials.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

We do not currently have patent protection for certain of our products and product candidates. If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, the value of our technology and products will be adversely affected.

Our success will depend in large part on our ability to obtain and maintain protection in the United States and other countries for the intellectual property covering, or incorporated into, our technology and products. The patent situation in the field of biotechnology and pharmaceuticals generally is highly uncertain and involves complex legal, technical, scientific and factual questions. We may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection. Fosmetpantotate is covered by our U.S. Patent No. 8,673,883, which was granted in 2014 and expires in 2033. In addition, our U.S. Patent No. 9,181,286, which was granted on November 10,

2015 and expires in 2033, covers the use of fosmetpantotenate for the treatment of PKAN, and our U.S. Patent No. 9,629,862, which was granted on April 25, 2017 and also expires in 2033, covers pharmaceutical compositions that contain fosmetpantotenate. Sparsentan is covered by U.S. Patent No. 6,638,937, which expires in 2019 and to which we have an exclusive license. In addition, U.S. Patent No. 9,662,312, to which we also have an exclusive license and which was granted on May 30, 2017 and expires in 2030, covers the use of sparsentan for treating glomerulosclerosis, including FSGS. And U.S. Patent No.9,993,461, to which we also have an exclusive license and which was granted on June 12, 2018 and expires in 2030, covers the use of sparsentan for treating IgA nephropathy as well as glomerulosclerosis, including FSGS.

For products we develop based on a new chemical entity not previously approved by the FDA, we expect that in addition to the protection afforded by our patent filings that we will be able to obtain either five years regulatory exclusivity via the provisions of the FDC Act and possibly seven years regulatory exclusivity via the orphan drug provisions of the FDC Act. In addition, we may be able to obtain up to five years patent term extension (to compensate for regulatory approval delay) for a patent covering such a product.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents issued to us or our licensors that provide a basis for commercially viable products will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies that are patentable;
- we will file patent applications for new proprietary technologies promptly or at all;
- the claims we make in our patents will be upheld by patent offices in the United States and elsewhere;
- our patents will not expire prior to or shortly after commencing commercialization of a product; and
- the patents of others will not have a negative effect on our ability to do business.

We have negotiated a license agreement with Ligand Pharmaceuticals for the rights to sparsentan which we are initially developing for the treatment of FSGS and IgAN. This license subjects us to various commercialization, reporting and other obligations. If we were to default on our obligations, we could lose our rights to sparsentan. We have obtained a U.S. and European patent covering the use of sparsentan for treating glomerulosclerosis, including FSGS, and a second U.S. patent covering both the use of sparsentan for treating IgAN and the use of sparsentan for treating glomerulosclerosis, including FSGS. However, we cannot be certain that we will be able to obtain patent protection for various other potential indications for sparsentan, or whether, if granted, we would be able to enforce such patents.

Our patents also may not afford us protection against competitors with similar technology. Because patent applications in the United States and many other jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind the actual discoveries, neither we nor our licensors can be certain that we or they were the first to make the inventions claimed in our or their issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications. If a third party has also filed a United States patent application prior to the effective date of the relevant provisions of the America Invents Act (i.e. before March 16, 2013) covering our product candidates or a similar invention, we may have to participate in an adversarial proceeding, known as an interference, declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial and it is possible that our efforts could be unsuccessful, resulting in a loss of our United States patent position.

We cannot assure you that third parties will not assert patent or other intellectual property infringement claims against us with respect to technologies used in our products. If patent infringement suits were brought against us, we may be unable to commercialize some of our products which could severely harm our business. Litigation proceedings, even if not successful, could result in substantial costs and harm our business.

We expect to rely on orphan drug status to develop and commercialize certain of our product candidates, but our orphan drug designations may not confer marketing exclusivity or other expected commercial benefits.

We expect to rely on orphan drug exclusivity for fosmetpantotenate and sparsentan and potential future product candidates that we may develop. Orphan drug status currently confers seven years of marketing exclusivity in the United States under the FDC Act, and up to ten years of marketing exclusivity in Europe for a particular product in a specified indication. The FDA and EMA have granted orphan designation for Chenodal, fosmetpantotenate and sparsentan for the treatment of CTX, PKAN and FSGS, respectively. While we have been granted these orphan designations, we will not be able to rely on these designations to exclude other companies from manufacturing or selling these molecules for the same indication beyond these time frames. Furthermore, any marketing exclusivity in Europe can be reduced from ten years to six years if the initial designation criteria have significantly changed since the market authorization of the orphan product.

For any product candidate for which we have been granted orphan drug designation in a particular indication, it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity expires. Even if we are the first to obtain marketing authorization for an orphan drug indication in the United States, there are circumstances under which a competing product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be

clinically superior to our orphan product, or if the later product is deemed a different product than ours. Further, the seven-year marketing exclusivity would not prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation, or for the use of other types of products in the same indications as our orphan product.

Any drugs we develop may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, thereby harming our business.

In March 2010, President Obama signed the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The PPACA revised the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. The PPACA also increased the mandated Medicaid rebate from 15.1% to 23.1% of the average manufacturer price, expanded the rebate to Medicaid managed care utilization and increased the types of entities eligible for the federal 340B drug discount program. Further, the law imposes a significant annual fee on companies that manufacture or import certain branded prescription drug products. There have been judicial, Congressional, and political challenges to certain aspects of the PPACA, as well as recent efforts by the Trump administration to repeal or replace certain aspects of the PPACA. Since January 2017, President Trump has signed two Executive Orders and other directives designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the PPACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the PPACA have been signed into law. The Tax Act of 2017 includes a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". On January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018 ("BBA"), among other things, amends the PPACA, effective January 1, 2019, to increase the discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D from 50 percent to 70 percent, and closes the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole". In July 2018, the Centers for Medicare and Medicaid Services ("CMS") published a final rule permitting further collections and payments to and from certain PPACA qualified health plans and health insurance issuers under the PPACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. On December 14, 2018, a Texas U.S. District Court Judge ruled that the PPACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act of 2017. While the Texas U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the PPACA will impact the PPACA and our business.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. For example, in August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least \$1.2 trillion for fiscal years 2012 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments, including the BBA, will stay in effect through 2027 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals and imaging centers.

If we are unable to obtain coverage and adequate reimbursement from governments or third-party payers for any products that we may develop or if we are unable to obtain acceptable prices for those products, our prospects for generating revenue and achieving profitability will suffer.

Our prospects for generating revenue and achieving profitability will depend heavily upon the availability of coverage and adequate reimbursement for the use of our approved product candidates from governmental and other third-party payers, both in the United States and in other markets. Reimbursement by a third-party payer may depend upon a number of factors, including the third-party payer's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining reimbursement approval for a product from each government or other third-party payer is a time consuming and costly process that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to each payer. We may not be able to provide data sufficient to gain acceptance with respect to reimbursement or we might need to conduct post-marketing studies in order to demonstrate the cost-effectiveness of any future products to such payers' satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources. Even when a payer determines that a product is eligible for reimbursement, the payer may impose coverage limitations that preclude payment for some uses that are approved by the FDA or non-United States regulatory authorities. Also

prior authorization for a product may be required. In addition, there is a risk that full reimbursement may not be available for high-priced products. Moreover, eligibility for coverage does not imply that any product will be reimbursed in all cases or at a rate that allows us to make a profit or even cover our costs. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent.

A primary trend in the United States healthcare industry and elsewhere is toward cost containment. We expect the changes made by PPACA, other legislation impacting the Medicare program and the 340B program, and the increasing emphasis on managed care to continue to put pressure on pharmaceutical product pricing. As these concerns continue to grow over the need for tighter oversight, there remains the possibility that the Health Resources and Services Administration or another agency under the HHS will propose regulations or that Congress will explore changes to the 340B program through legislation. For example, a bill was introduced in 2018 that would require hospitals to report their low-income utilization of the program. Further, the Centers for Medicare & Medicaid Services issued a final rule that would revise the Medicare hospital outpatient prospective payment system for calendar year 2019, including a new reimbursement methodology for drugs purchased under the 340B program for Medicare patients at the hospital setting and recently announced the same change for physician-based practices under 340B in 2019. In addition, HHS has set January 1, 2019, as the effective date of the final rule setting forth the calculation of the ceiling price and application of civil monetary penalties. Pursuant to the final rule, after January 1, 2019, manufacturers must calculate 340B program ceiling prices on a quarterly basis. Moreover, manufacturers could be subject to a \$5,000 penalty for each instance where they knowingly and intentionally overcharge a covered entity under the 340B program.

Further, there has been increasing legislative and enforcement interest in the United States with respect to drug pricing practices, including several recent U.S. congressional inquiries and proposed federal and enacted state legislation designed to, among other things, increase drug pricing transparency, expedite generic competition, review relationships between pricing and manufacturer patient assistance programs, and reform government program drug reimbursement methodologies. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Further, the current administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The HHS has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. On January 31, 2019, the HHS Office of Inspector General proposed modifications to the Federal Anti-Kickback Statute discount safe harbor for the purpose of reducing the cost of drug products to consumers which, among other things, if finalized, will affect discounts paid by manufacturers to Medicare Part D plans, Medicaid managed care organizations and pharmacy benefit managers working with these organizations. Although a number of these, and other potential proposals, may require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Any reduction in reimbursement from Medicare, Medicaid or other government-funded programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs. Additionally, we are currently unable to predict what additional legislation or regulation, if any, relating to the healthcare industry may be enacted in the future or what effect recently enacted federal legislation or any such additional legislation or regulation would have on our business.

We face potential product liability exposure far in excess of our limited insurance coverage.

The use of any of our potential products in clinical trials, and the sale of any approved products, may expose us to liability claims. These claims might be made directly by consumers, health care providers, pharmaceutical companies or others selling our products. We have obtained limited product liability insurance coverage for our clinical trials in the amount of \$10 million per occurrence and \$10 million in the aggregate. However, our insurance may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. We intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. On occasion, juries have awarded large judgments in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us would decrease our cash reserves and could cause our stock price to fall.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do. Our operating results will suffer if we fail to compete effectively.

Several of our competitors have substantially greater financial, research and development, distribution, manufacturing and marketing experience and resources than we do and represent substantial long-term competition for us. Other companies may succeed in developing and marketing products that are more effective and/or less costly than any products that may be developed and marketed by us, or that are commercially accepted before any of our products. Factors affecting competition in the pharmaceutical and drug industries vary, depending on the extent to which a competitor is able to achieve a competitive advantage based on its proprietary technology and ability to market and sell drugs. The industry in which we compete is characterized by extensive research and development efforts and rapid technological progress. Although we believe that our orphan drug status for Cholbam and proprietary position with respect to fosmetpantotenate and sparsentan may give us a competitive advantage, new developments are expected to continue and there can be no assurance that discoveries by others will not render such potential products noncompetitive. Furthermore, competitors could enter the market with generic versions of our products.

Our competitive position also depends on our ability to enter into strategic alliances with one or more large pharmaceutical and contract manufacturing companies, attract and retain qualified personnel, develop effective proprietary products, implement development and marketing plans, obtain patent protection, secure adequate capital resources and successfully sell and market our approved products. There can be no assurance that we will be able to successfully achieve all of the foregoing objectives.

Use of third parties to manufacture and distribute our products and product candidates may increase the risk that we will not have sufficient quantities of our product and product candidates or such quantities at an acceptable cost, and clinical development and commercialization of our product and product candidates could be delayed, prevented or impaired.

We do not own or operate manufacturing facilities for clinical or commercial production of our products. We have limited personnel with experience in drug manufacturing and we lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We outsource all manufacturing and packaging of our preclinical, clinical, and commercial products to third parties. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production and in maintaining required quality control. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate.

We do not currently have any agreements with third-party manufacturers for the long-term commercial supply of any of our development stage product candidates. We may be unable to enter into agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms. Even if we enter into these agreements, the manufacturers of each product candidate will be single source suppliers to us for a significant period of time. Reliance on third-party manufacturers entails risks to which we may not be subject if we manufactured our product candidates or products ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using our products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

Our contract manufacturers will be required to adhere to FDA regulations setting forth cGMP. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our manufacturers are subject to unannounced inspections by the FDA, state regulators and similar regulators outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. If the third parties that we engage to manufacture products for our developmental or commercial products should cease to continue to do so for any reason, we likely would experience interruptions in cash flows and/or delays in advancing our clinical trials while we identify and qualify replacement suppliers, and we may be unable to obtain replacement supplies on terms that are favorable to us. Later relocation to another manufacturer will also require notification, review and other regulatory approvals from the FDA and other regulators and will subject our production to further cost and instability in the availability of our product candidates. In addition, if we are not able to obtain adequate supplies of our product

candidates, or the drug substances used to manufacture them, it will be more difficult for us to sell our products and to develop our product candidates. This could greatly reduce our competitiveness.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that obtain regulatory approval on a timely and competitive basis.

Materials necessary to manufacture our products and product candidates may not be available on commercially reasonable terms, or at all, which may delay the development and commercialization of our products and product candidates.

We rely on the manufacturers of our products and product candidates to purchase from third-party suppliers the materials necessary to produce the compounds for our preclinical and clinical studies and rely on these other manufacturers for commercial distribution if we obtain marketing approval for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls, and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our preclinical and clinical studies, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product candidates.

Risks Related to Our Business

Our limited operating history makes it difficult to evaluate our future prospects, and our profitability in the future is uncertain.

We face the problems, expenses, difficulties, complications and delays, many of which are beyond our control, associated with any business in its early stages and have a limited operating history on which an evaluation of our prospects can be made. Such prospects should be considered in light of the risks, expenses and difficulties frequently encountered in the establishment of a business in a new industry, characterized by a number of market entrants and intense competition, and in the shift from development to commercialization of new products based on innovative technologies.

We have experienced significant growth over the past three years in the number of our employees and the scope of our operations. We have added sales and marketing, compliance and legal functions in addition to expansion of all functions to support a commercial organization. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

Factors that may inhibit our efforts to commercialize our products without strategic partners or licensees include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or educate adequate numbers of physicians to prescribe our products;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;
- unforeseen costs associated with expanding our own sales and marketing team for new products or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize competitive products.

Moreover, though we generate revenues from product sales arrangements, we may incur significant operating losses over the next several years. Our ability to achieve profitable operations in the future will depend in large part upon successful in-licensing of products approved by the FDA, selling and manufacturing these products, completing development of our products, obtaining regulatory approvals for these products, and bringing these products to market. The likelihood of the long-term success of our company must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new drug products, competitive factors in the marketplace, as well as the regulatory environment in which we operate.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors.

*** We will likely experience fluctuations in operating results and could incur substantial losses.**

We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and preclinical development activities. We have not completed development of any drugs and we anticipate that our expenses will increase substantially as we:

- continue our ongoing clinical development of fosmetpantotenate for the treatment of PKAN;

- continue the open label portion of DUET and conduct the planned Phase 3 trials of sparsentan indications;
- continue funding the clinical development of CNSA-001 for PKU;
- assuming FDA approval, the commercial launch of the next generation of Thiola;
- continue the research and development of additional product candidates;
- expand our sales and marketing infrastructure to commercialize our current products and any new products for which we may obtain regulatory approval; and
- expand operational, financial, and management information systems and personnel, including personnel to support product development efforts and our obligations as a public company.

To attain and sustain profitability, we must succeed in developing and commercializing drugs with significant market potential. This will require us to be successful in a range of challenging activities, including the discovery of product candidates, successful completion of preclinical testing and clinical trials of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain regulatory approval. We are only in the preliminary stages of these activities. We may not be successful enough in these activities to generate revenues that are substantial enough to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become or remain profitable could depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the market price of our common stock may also cause a loss of a part or all of your investment.

Negative publicity regarding any of our products could impair our ability to market any such product and may require us to spend time and money to address these issues.

If any of our products or any similar products distributed by other companies prove to be, or are asserted to be, harmful to consumers and/or subject to FDA enforcement action, our ability to successfully market and sell our products could be impaired. Because of our dependence on patient and physician perceptions, any adverse publicity associated with illness or other adverse effects resulting from the use or misuse of our products or any similar products distributed by other companies could limit the commercial potential of our products and expose us to potential liabilities.

We may not have sufficient insurance to cover our liability in any current or future litigation claims either due to coverage limits or as a result of insurance carriers seeking to deny coverage of such claims.

We face a variety of litigation-related liability risks. Our certificate of incorporation, bylaws, other applicable agreements, and/or Delaware law require us to indemnify (and advance expenses to) our current and past directors and officers and employees from reasonable expenses related to the defense of any action arising from their service to us, including circumstances under which indemnification is otherwise discretionary. While our directors and officers are included in a director and officer liability insurance policy, which covers all our directors and officers in some circumstances, our insurance coverage does not cover all of our indemnification obligations and may not be adequate to cover any indemnification or other claims against us. In addition, the underwriters of our present coverage may seek to avoid coverage in certain circumstances based upon the terms of the respective policies. If we incur liabilities that exceed our coverage under our directors and officers insurance policy or incur liabilities not covered by our insurance, we would have to self-fund any indemnification amounts owed to our directors and officers and employees in which case our results of operations and financial condition could be materially adversely affected. Further, if D&O insurance becomes prohibitively expensive to maintain in the future, we may be unable to renew such insurance on economic terms or unable to renew such insurance at all. The lack of D&O insurance may make it difficult for us to retain and attract talented and skilled directors and officers to serve our company, which could adversely affect our business.

*** We may need substantial funding and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs or commercialization efforts.**

We expect our general and research and development expenses to increase in connection with our ongoing and planned activities, particularly as we conduct Phase 3 clinical trials of fosmetpantotenate and sparsentan, continue funding the clinical development of CNSA-001 and potentially acquire Censa, and conduct any other later-stage clinical trials of our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers, and product distribution. We currently have no additional commitments or arrangements for any additional financing to fund the research and development and commercial launch of our product candidates.

Management believes our ability to continue our operations depends on our ability to sustain and grow revenue, results of operations and our ability to access capital markets when necessary to accomplish our strategic objectives. Management believes that we may incur losses in the immediate future. We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and preclinical development activities. We expect to finance our cash needs from cash on hand and results of operations, and depending on results of operations we may either need additional equity or debt financing, or need to enter into strategic alliances on products in development to continue our operations until we can achieve sustained profitability and positive cash flows from operating activities. Additional funds may not be available to us when we need them on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.

Our future capital requirements will depend on many factors, including:

- the progress and results of our pre-clinical and clinical studies of fosmetpantotenate, sparsentan, CNSA-001 and other drug candidates;

- the costs, timing and outcome of regulatory review of our product candidates;
- debt service obligations on the 2019 Notes, which mature on May 30, 2019, and 2025 Notes;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies, including the extent to which we exercise our option to acquire Censa; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

The market price for shares of our common stock may be volatile and purchasers of our common stock could incur substantial losses.

The price of our stock is likely to be volatile. The stock market in general, and the market for biotechnology companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our product candidates or those of our competitors;
- our entry into or the loss of a significant collaboration;
- regulatory or legal developments in the United States and other countries, including changes in the health care payment systems;
- our ability to obtain and maintain marketing approvals from the FDA or similar regulatory authorities outside the United States;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;
- general economic, industry and market conditions;
- results of clinical trials conducted by others on drugs that would compete with our product candidates;
- developments or disputes concerning patents or other proprietary rights;
- public concern over our product candidates or any products approved in the future;
- litigation;
- communications from government officials regarding health care costs or pharmaceutical pricing;
- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this "Risk Factors" section.

In addition, the stock markets, and in particular, the Nasdaq Global Market, have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many pharmaceutical companies. The realization of any of the above risks or any of a broad range of other risks, including those described in these "Risk Factors" could have a dramatic and material adverse impact on the market price of our common stock.

We may be unable to successfully integrate new products or businesses we may acquire.

We intend to expand our product pipeline by pursuing acquisition of pharmaceutical products. If an acquisition is consummated, including the extent to which we exercise our option to acquire Censa, the integration of the acquired business, product or other assets into our company may also be complex and time-consuming and, if such businesses, products and assets are not successfully integrated, we may not achieve the anticipated benefits, cost-savings or growth opportunities.

Potential difficulties that may be encountered in the integration process include the following:

- integrating personnel, operations and systems, while maintaining focus on producing and delivering consistent, high quality products;
- coordinating geographically dispersed organizations;
- distracting employees from operations;
- retaining existing customers and attracting new customers; and
- managing inefficiencies associated with integrating the operations of the Company.

Furthermore, these acquisitions and other arrangements, even if successfully integrated, may fail to further our business strategy as anticipated, expose us to increased competition or challenges with respect to our products or geographic markets, and expose us to additional liabilities associated with an acquired business, product, technology or other asset or arrangement. Any one of these challenges or risks could impair our ability to realize any benefit from our acquisitions or arrangements after we have expended resources on them.

If we are unable to maintain an effective and specialized sales force, we will not be able to commercialize our products successfully.

In order to successfully commercialize our products, we have built a specialized sales force. Factors that may hinder our ability to successfully market and commercially distribute our products include:

- inability of sales personnel to obtain access to or convince adequate numbers of physicians to prescribe our products;
- inability to recruit, retain and effectively manage adequate numbers of effective sales personnel;
- lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies that have more extensive product lines; and
- unforeseen delays, costs and expenses associated with maintaining our sales organization.

If we are unable to maintain our sales force for our products, we may not be able to generate sufficient product revenue.

We will need to continue to expend significant time and resources to train our sales forces to be credible, persuasive and compliant in discussing our products with the specialists treating the patients indicated under the product's label. In addition, if we are unable to effectively train our sales force and equip them with effective marketing materials our ability to successfully commercialize our products could be diminished, which would have a material adverse effect on our business, results of operations and financial condition.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

Our business exposes us to potential liability risks inherent in the research, development, manufacturing and marketing of pharmaceutical products. If any of our product candidates in clinical trials or commercialized products harm people we may be subject to costly and damaging product liability claims. We have clinical trial insurance and commercial product liability coverage. However, this insurance may not be adequate to cover all claims. We may be exposed to product liability claims and product recalls, including those which may arise from misuse or malfunction of, or design flaws in, such products, whether or not such problems directly relate to the products and services we have provided. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- damage to our reputation;
- regulatory investigations that could require costly recalls or product modifications;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;
- loss of revenue;
- the diversion of management's attention from managing our business; and
- the inability to commercialize any products that we may develop.

A successful product liability claim or a series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our available cash and adversely affect our business.

We are involved in certain litigation matters, any of which could result in substantial costs, divert management's attention and otherwise have a material adverse effect on our business, operating results or financial condition.

We are involved in certain litigation matters, including those described in Note 12 of the Consolidated Financial Statements included in this report. Although we intend to vigorously defend our interests in each matter, there is no guarantee that we will be successful and we may have to pay damages awards or otherwise may enter into settlement arrangements in connection with such matters. Any such payments or settlement arrangements could have material adverse effects on our business, operating results or financial condition. Even if we are successful in defending our interests in each matter, litigation with respect to such matters could result in substantial costs and significant adverse impact on our reputation and divert management's attention and resources, which could have a material adverse effect on our business, operating results or financial condition.

We are subject to significant ongoing regulatory obligations and oversight, which may result in significant additional expense and may limit our commercial success.

We are subject to significant ongoing regulatory obligations, such as safety reporting requirements and additional post-marketing obligations, including regulatory oversight of the promotion and marketing of our products. In addition, the manufacture, quality control, labeling, packaging, safety surveillance, adverse event reporting, storage and recordkeeping for our products are subject to extensive and ongoing regulatory requirements. If we become aware of previously unknown problems with any of our products, a regulatory agency may impose restrictions on our products, our contract manufacturers or us. If we, our products and product candidates, or the manufacturing facilities for our products and product candidates fail to comply with applicable regulatory requirements, a regulatory agency, including the FDA, may send enforcement letters, mandate labeling changes, suspend or withdraw regulatory approval, suspend any ongoing clinical trials, refuse to approve pending applications or supplements filed by us, suspend or impose restrictions on manufacturing operations, request a recall of, seize or detain a product, seek criminal prosecution or an injunction, or impose civil or criminal penalties or monetary fines. In such instances, we could experience a significant drop in the sales of the affected products, our product revenues and reputation in the marketplace may suffer, and we could become the target of lawsuits.

We are also subject to regulation by national, regional, state and local agencies, including but not limited to the FDA, CMS, Department of Justice, the Federal Trade Commission, the HHS Office of Inspector General and other regulatory bodies. The FDC Act, Social Security Act, Public Health Service Act and other federal and state statutes and regulations govern to varying degrees the research, development, manufacturing and commercial activities relating to prescription pharmaceutical products, including preclinical testing, clinical research, approval, production, labeling, sale, distribution, post-market surveillance, advertising, dissemination of information, promotion, marketing, and pricing to government purchasers and government health care programs. Our manufacturing partners are subject to many of the same requirements.

Companies may not promote drugs for “off-label” uses—that is, uses that are not described in the product’s labeling and that differ from those approved by the FDA or other applicable regulatory agencies. However, a company may share truthful and not misleading information that is otherwise consistent with the product’s labeling. A company that is found to have improperly promoted off-label uses may be subject to significant liability, including civil and administrative remedies as well as criminal sanctions. In addition, management’s attention could be diverted from our business operations and our reputation could be damaged.

The federal health care program Anti-Kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting, or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any health care item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements that pharmaceutical companies have with prescribers, purchasers and formulary managers, among others. Further, the PPACA, among other things, amends the intent requirement of the federal anti-kickback statute so that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act. Although there are a number of statutory exceptions and regulatory safe harbors under the federal anti-kickback statute protecting certain common manufacturer business arrangements and activities from prosecution, the exceptions and safe harbors are drawn narrowly and an arrangement must meet all of the conditions specified in order to be fully protected from scrutiny under the federal anti-kickback statute. We seek to comply with the exceptions and safe harbors whenever possible, but our practices, such as our patient assistance programs and prompt pay discounts with certain customers, may not in all cases meet all of the criteria for protection from anti-kickback liability and may be subject to scrutiny.

The federal false claims laws, including the federal False Claims Act, prohibit any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to get a false claim paid. Additionally, the civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent. Many pharmaceutical and other health care companies have been investigated and have reached substantial financial settlements with the federal government under the federal False Claims Act for a variety of alleged marketing activities, including providing free product to customers with the expectation that the customers would bill federal programs for the product; providing consulting fees, grants, free travel, and other benefits to physicians to induce them to prescribe the company’s products; and inflating prices reported to private price publication services, which may be used by states to set drug payment rates under government health care programs. Companies have been prosecuted for causing false claims to be submitted because of the marketing of their products for unapproved uses. Pharmaceutical and other health care companies have also been prosecuted on other legal theories of Medicare and Medicaid fraud.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. It is not clear whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of any Retrophin products, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject Retrophin to more stringent product labeling and post-marketing testing and other requirements.

We also could become subject to government investigations and related subpoenas. Such subpoenas are often associated with previously filed qui tam actions, or lawsuits filed under seal under the federal False Claims Act. Qui tam actions are brought by private plaintiffs suing on behalf of the federal government for alleged violations of the federal False Claims Act. The time and expense associated with responding to such subpoenas, and any related qui tam or other actions, may be extensive, and we cannot predict the results of our review of the responsive documents and underlying facts or the results of such actions. Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties,

could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being added to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular, the PPACA includes a number of provisions aimed at strengthening the government's ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, amendments to the federal False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations and, for payments made on or after August 1, 2013, public reporting of payments by pharmaceutical manufacturers to physicians and teaching hospitals nationwide. We anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of further government investigations and enforcement actions. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

The U.S. Foreign Corrupt Practices Act, and similar worldwide anti-bribery laws generally prohibit companies and their intermediaries from making improper payments to government officials for the purpose of obtaining or retaining business. Our policies mandate compliance with these anti-bribery laws. We operate in parts of the world that have experienced governmental corruption to some degree and in certain circumstances, strict compliance with antibribery laws may conflict with local customs and practices or may require us to interact with doctors and hospitals, some of which may be state controlled, in a manner that is different than in the United States. We cannot assure that our internal control policies and procedures will protect us from reckless or criminal acts committed by our employees or agents. Violations of these laws, or allegations of such violations, could disrupt our business and result in criminal or civil penalties or remedial measures, any of which could have a material adverse effect on our business, financial condition and results of operations and could cause the market value of our common stock to decline.

The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), created new federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payers, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like the federal anti-kickback statute, the PPACA amended the intent standard for certain healthcare fraud provisions under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

Additionally, the federal Physician Payments Sunshine Act within the PPACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biologicals and medical supplies to report annually information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members. Moreover, the Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

Also, many states have similar fraud and abuse statutes or regulations, including state anti-kickback and false claims laws, that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Further, certain states require implementation of commercial compliance programs and marketing codes, compliance with the pharmaceutical industry's voluntary compliance guidelines, and compliance with the applicable compliance guidance promulgated by the federal government. Other various state level requirements include restricting payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; restricting various marketing practices; requiring prescription drug companies to report expenses relating to the marketing and promotion of drug products; requiring the posting of information relating to clinical studies and their outcomes; requiring the registration of sales representatives; requiring the reporting of certain information related to drug pricing; and requiring drug manufacturers to track and report information related to payments, gifts, compensation, and other items of value to physicians and other healthcare providers.

In addition, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, imposes specified requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH, through its implementing regulations, makes certain of HIPAA's privacy and security standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains or transmits protected health information for or on behalf of a covered entity for a function or activity regulated by HIPAA. International data protection laws also impose strict obligations on the ability to process health related and other personal information of citizens of member states, including in relation to collection, analysis and transfer. The EU General Data Protection Regulation was officially adopted in April 2016 and has been in effect since May 2018. The EU General Data Protection Regulation introduced new data protection requirements in the European Union, as well as substantial fines for breaches of the data protection rules. The EU General Data Protection Regulation will increase our responsibility and liability in relation to personal data that we process, and we may be required to put in place additional mechanisms to ensure compliance with the new EU data protection rules. Additionally, California recently enacted legislation known as the California Consumer Privacy Act (the "CCPA"), which creates new individual privacy rights for consumers (as that word is broadly defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. When it goes into effect on January 1, 2020, the CCPA will require covered companies to provide new disclosures to California consumers, provide such consumers new ways to opt-out of certain sales of personal information, and allow for a new cause of action for data breaches. Legislators have stated that amendments will be proposed to the CCPA before it goes into effect, but it remains unclear what, if any, modifications will be made to this legislation or how it will be interpreted. As currently

written, the CCPA will likely impact (possibly significantly) our business activities and exemplifies the vulnerability of our business to not only cyber threats but also the evolving regulatory environment related to personal data and protected health information.

If we or any of our partners fail to comply with applicable regulatory requirements, we or they could be subject to a range of regulatory actions that could affect our or our partners' ability to commercialize our products and could harm or prevent sales of the affected products, or could substantially increase the costs and expenses of commercializing and marketing our products. Any threatened or actual government enforcement action could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Compliance with applicable federal and state laws is difficult and time consuming, and companies that violate them may face substantial penalties. The potential sanctions include criminal fines, civil monetary penalties, administrative penalties, disgorgement, exclusion from participation in federal health care programs, individual imprisonment, injunctions, recall or seizure of products, total or partial suspension of production, reputational harm, administrative burdens, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegation of non-compliance with these laws, diminished profits and future earnings, and the curtailment or restructuring of our operations, and other sanctions. Because of the breadth of these laws, it is possible that some of our business activities could be subject to challenge under one or more of these laws. Such a challenge, irrespective of the underlying merits of the challenge or the ultimate outcome of the matter, could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

If we are not able to obtain and maintain required regulatory approvals, we will not be able to commercialize our products, and our ability to generate revenue will be materially impaired.

Our product candidates, once approved, and the activities associated with their manufacture, marketing, distribution, and sales are subject to extensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to adhere to regulations set out by these bodies for one or more of our commercial products could prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in meeting the regulatory requirements incumbent on the sale of drugs in the United States and elsewhere, and expect to rely on third-parties to assist us in these processes. If these third parties fail to adequately adhere to the regulations governing drug distribution and promotion we may be unable to sell our products, which could have a material effect on our ability to generate revenue.

Our product candidates and the activities associated with their development and commercialization, including testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain regulatory approval for a product candidate will prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in filing and prosecuting the applications necessary to obtain regulatory approvals and expect to rely on third-party contract research organizations to assist us in this process.

Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish the product candidate's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and successful inspection of manufacturing facilities by, the FDA. Our future products may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

Our product candidates may fail to obtain regulatory approval for many reasons, including:

- our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable regulatory authorities for approval;
- our inability to demonstrate that a product candidate's benefits outweigh its risks;
- our inability to demonstrate that the product candidate presents an advantage over existing therapies;
- the FDA's or comparable regulatory authorities' disagreement with the manner in which we interpret the data from preclinical studies or clinical trials;
- failure of the third-party manufacturers with which we contract for clinical or commercial supplies to satisfactorily complete an FDA pre-approval inspection of the facility or facilities at which the product is manufactured to assess compliance with the FDA's cGMP regulations to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and
- a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. The FDA and non-United States regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing

could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

Our internal computer systems, or those of our CROs or other contractors and vendors who host our applications or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Despite the implementation of security measures, our internal computer systems and those of our CROs and other contractors or vendors who host our applications and those of our consultants are vulnerable to damage or disruption from computer viruses, software bugs, unauthorized access including cyber-attack, natural disasters, terrorism, war, and telecommunication, equipment and electrical failures. While we have not, to our knowledge, experienced any significant system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data from completed or ongoing clinical trials for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure or theft of confidential or proprietary information, we could incur liability, the further development of our product candidates could be delayed, our competitive position could be compromised, or our business reputation could be harmed.

*** Comprehensive tax reform could adversely affect our business and financial condition.**

On December 22, 2017, President Trump signed into law the Tax Act of 2017 that significantly revises the Internal Revenue Code of 1986, as amended. The Tax Act of 2017, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted taxable income (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and eliminating most carrybacks of net operating losses arising in taxable years ending after December 31, 2017, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the Tax Act of 2017 is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the Tax Act of 2017. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to the Tax Act of 2017 and the potential tax consequences of investing in or holding our common stock.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

Our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors, including passage of the Tax Act of 2017, changes in the mix of our profitability from state to state, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

Our ability to use net operating losses to offset future taxable income may be subject to limitations.

As of December 31, 2018, we had federal net operating loss, or NOL, carryforwards of \$36.5 million. Our federal NOL carryforwards generated in tax years ending on or prior to December 31, 2017, are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under the Tax Act of 2017, our federal NOLs generated in tax years ending after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOLs generated in tax years beginning after December 31, 2017, is limited. It is uncertain if and to what extent various states will conform to the Tax Act of 2017. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation’s ability to use its pre-change NOL carryforwards and other pre-change U.S. tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. It is possible that we have experienced an ownership change limitation in the past. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control.

As a result, our pre-2018 NOL carryforwards may expire prior to being used, and our NOL carryforwards generated in 2018 and thereafter will be subject to a percentage limitation. In addition, it is possible that we have in the past undergone, and in the future may undergo, additional ownership changes that could limit our ability to use all of our pre-change NOLs and other pre-change tax attributes (such as research tax credits) to offset our post-change income or taxes. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. As a result, we may be unable to use all or a material portion of our NOLs and other tax attributes, which would harm our future operating results by effectively increasing our future tax obligations.

We are dependent on information technology systems, infrastructure and data, which exposes us to data security risks.

We are dependent upon our own or third-party information technology systems, infrastructure and data, including mobile technologies, to operate our business. The multitude and complexity of our computer systems may make them vulnerable to service interruption or destruction, disruption of

data integrity, malicious intrusion, or random attacks. Likewise, data privacy or security incidents or breaches by employees or others may pose a risk that sensitive data, including our intellectual property, trade secrets or personal information of our employees, patients, customers or other business partners may be exposed to unauthorized persons or to the public. Cyber-attacks are increasing in their frequency, sophistication and intensity. Cyber-attacks could include the deployment of harmful malware, denial-of-service, social engineering and other means to affect service reliability and threaten data confidentiality, integrity and availability. Our business partners face similar risks and any security breach of their systems could adversely affect our security posture. A security breach or privacy violation that leads to disclosure or modification of or prevents access to patient information, including personally identifiable information or protected health information, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, require us to verify the correctness of database contents and otherwise subject us to litigation or other liability under laws and regulations that protect personal data, any of which could disrupt our business and/or result in increased costs or loss of revenue. Moreover, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information, trade secrets or other intellectual property. While we have invested, and continue to invest, in the protection of our data and information technology infrastructure, there can be no assurance that our efforts will prevent service interruptions, or identify breaches in our systems, that could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business or reputational harm to us. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyber-attacks and other related breaches.

Risks Related to our Indebtedness and Investments

* Our indebtedness could adversely affect our financial condition.

As of March 31, 2019, we had approximately \$298.6 million of total debt outstanding, classified as short and long term. As a result of our indebtedness, a portion of our cash flow will be required to pay interest and principal on the 2019 Notes, which mature on May 30, 2019, and 2025 Notes if the notes are not converted to shares of common stock prior to maturity. We may not generate sufficient cash flow from operations or have future borrowings available to enable us to repay our indebtedness or to fund other liquidity needs.

Our indebtedness pursuant to the 2019 Notes and 2025 Notes could have important consequences. For example, it could:

- make it more difficult for us to satisfy our obligations with respect to any other debt we may incur in the future;
- increase our vulnerability to general adverse economic and industry conditions;
- require us to dedicate a substantial portion of our cash flow from operations to payments on our indebtedness and related interest, thereby reducing the availability of our cash flow to fund working capital, capital expenditures and other general corporate purposes;
- limit our flexibility in planning for, or reacting to, changes in our business and the industry in which we operate;
- increase our cost of borrowing;
- place us at a competitive disadvantage compared to our competitors that may have less debt; and
- limit our ability to obtain additional financing for working capital, capital expenditures, acquisitions, debt service requirements or general corporate purposes.

We expect to use cash flow from operations and outside financings to meet our current and future financial obligations, including funding our operations, debt service and capital expenditures. Our ability to make these payments depends on our future performance, which will be affected by financial, business, economic and other factors, many of which we cannot control. Our business may not generate sufficient cash flow from operations in the future, which could result in our being unable to repay indebtedness, or to fund other liquidity needs. If we do not generate sufficient cash from operations, we may be forced to reduce or delay our business activities and capital expenditures, sell assets, obtain additional debt or equity capital or restructure or refinance all or a portion of our debt, including the 2019 Notes and 2025 Notes, on or before maturity. We cannot make any assurances that we will be able to accomplish any of these alternatives on terms acceptable to us, or at all. In addition, the terms of existing or future indebtedness may limit our ability to pursue any of these alternatives.

We may be unable to raise the funds necessary to repurchase the 2019 Notes and 2025 Notes for cash following a fundamental change, or to pay any cash amounts due upon conversion, and our future indebtedness may limit our ability to repurchase the 2019 Notes and 2025 Notes or pay cash upon their conversion.

Noteholders may require us to repurchase their 2025 Notes following a fundamental change at a cash repurchase price generally equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date. In addition, upon conversion, we will satisfy part or all of our conversion obligation in cash unless we elect to settle conversions solely in shares of our common stock.

Further, the indenture governing the 2019 Notes contains a similar definition of fundamental change, and noteholders of the 2019 Notes will likely have the right to require us to repurchase their 2019 Notes for cash upon the occurrence of a fundamental change at a repurchase price equal to 100% of the principal amount of the 2019 Notes to be repurchased, plus accrued and unpaid interest, if any.

We may not have enough available cash or be able to obtain financing at the time we are required to repurchase the 2025 Notes and/or the 2019 Notes or pay the cash amounts due upon conversion of the 2025 Notes. In addition, applicable law, regulatory authorities and the agreements

governing our future indebtedness may restrict our ability to repurchase the 2025 Notes and/or the 2019 Notes or pay the cash amounts due upon conversion of the 2025 Notes. Our failure to repurchase the 2025 Notes and/or the 2019 Notes or to pay the cash amounts due upon conversion of the 2025 Notes when required will constitute a default under the base and supplemental indentures that will govern the 2025 Notes, which we refer to collectively as the “indenture,” and/or a default under the indenture governing the 2019 Notes. A default under the indenture and/or the indenture governing the 2019 Notes or the fundamental change itself could also lead to a default under agreements governing our other indebtedness, which may result in that other indebtedness becoming immediately payable in full. We may not have sufficient funds to satisfy all amounts due under the other indebtedness, the 2019 Notes and the 2025 Notes.

A default under the 2025 Notes may have a material adverse effect on our financial condition.

If an event of default under the 2025 Notes occurs, the principal amount of the 2025 Notes, plus accrued and unpaid interest (including additional interest, if any) may be declared immediately due and payable, subject to certain conditions set forth in the indenture governing such notes. Events of default include, but are not limited to:

- failure to pay (for more than 30 days) interest when due;
- failure to pay principal when due;
- failure to deliver shares of common stock upon conversion of a 2025 Notes;
- failure to provide notice of a fundamental change;
- acceleration on our other indebtedness in excess of \$10 million (other than indebtedness that is non-recourse to us); or
- certain types of bankruptcy or insolvency involving us.

Accordingly, the occurrence of a default under the 2025 Notes, unless cured or waived, may have a material adverse effect on our results of operations.

Provisions of the 2019 Notes and 2025 Notes could discourage an acquisition of us by a third party.

Certain provisions of the 2019 Notes and 2025 Notes could make it more difficult or more expensive for or prevent a third party to acquire us. Upon the occurrence of certain transactions constituting a fundamental change, holders of the 2019 Notes and 2025 Notes will have the right, at their option, to require us to repurchase all of their 2019 Notes and 2025 Notes or any portion of the principal amount of such Notes in integral multiples of \$1,000. We may also be required to increase the conversion rate for conversions in connection with certain fundamental changes.

Conversion of the Notes may dilute the ownership interest of existing stockholders, including holders who had previously converted their 2019 Notes and 2025 Notes.

To the extent we issue shares of common stock upon conversion of the 2019 Notes and 2025 Notes, the conversion of some or all of the 2019 Notes and 2025 Notes will dilute the ownership interests of existing stockholders. Any sales in the public market of shares of the common stock issuable upon such conversion could adversely affect prevailing market prices of shares of our common stock. In addition, the existence of the 2019 Notes and 2025 Notes may encourage short selling by market participants because the conversion of the 2019 Notes and 2025 Notes could depress the price of shares of our common stock.

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

(a) Exhibits

3.1	Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to Amendment No. 2 to the Company's General Form for Registration of Securities on Form 10-12G, filed with the SEC on October 28, 2010).
3.2	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
3.3	Amended and Restated Bylaws of the Company (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
4.1	Form of Note Purchase Agreement for principal senior convertible notes with an interest rate of 4.50% due 2019 ("2019 Notes"), dated May 29, 2014, by and among the Company and the investors identified therein (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.2	Form of Indenture for 2019 Notes, dated May 30, 2014 (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.3	Form of Note for 2019 Notes, dated May 30, 2014 (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.4	Base Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
4.5	First Supplemental Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (including the form of 2.50% Convertible Senior Note due 2025) (incorporated by reference to Exhibit 4.2 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
10.1	Retirement Agreement, dated January 4, 2019, by and between the Company and Stephan Aselage (incorporated by reference to Exhibit 10.33 to the Company's Current Report on Form 10-K, filed with the SEC on February 26, 2019).
10.2	Employment Agreement, dated January 4, 2019, by and between the Company and Eric M. Dube (incorporated by reference to Exhibit 10.34 to the Company's Current Report on Form 10-K, filed with the SEC on February 26, 2019).
31.1	Chief Executive Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Chief Financial Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Chief Executive Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
32.2	Chief Financial Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Taxonomy Extension Presentation Linkbase Document

SIGNATURES

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

Date: May 7, 2019

RETROPHIN, INC.

By: /s/ Eric M. Dube

Name: Eric M. Dube

Title: Chief Executive Officer

By: /s/ Laura Clague

Name: Laura Clague

Title: Chief Financial Officer

CERTIFICATION OF CHIEF EXECUTIVE OFFICER
PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a)

I, Eric M. Dube, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Retrophin, 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 7, 2019

/s/ Eric M. Dube

Eric M. Dube

Chief Executive Officer

(Principal Executive Officer)

CERTIFICATION OF CHIEF FINANCIAL OFFICER
PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a)

I, Laura Clague, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Retrophin, 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 7, 2019

/s/ Laura Clague

Laura Clague

Chief Financial Officer

(Principle Financial Officer)

**CERTIFICATION OF
CHIEF EXECUTIVE OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350
AS ADOPTED PURSUANT TO**

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the accompanying Quarterly Report on Form 10-Q of Retrophin, Inc. (the "Company"), for the period ending March 31, 2019 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report, fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 7, 2019

/s/ Eric M. Dube

Eric M. Dube

Chief Executive Officer

(Principal Executive Officer)

**CERTIFICATION OF
CHIEF FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350
AS ADOPTED PURSUANT TO**

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the accompanying Quarterly Report on Form 10-Q of Retrophin, Inc. (the "Company"), for the period ending March 31, 2019 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report, fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 7, 2019

/s/ Laura Clague

Laura Clague

Chief Financial Officer

(Principal Financial Officer)