Retrophin, Inc.
Form 10-Q/A
July 06, 2018

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

_____

FORM 10-Q/A
Amendment No.1

_____

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

For the quarterly period ended March 31, 2018

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	27-4842691
(State or other jurisdiction of incorporation or organization)	(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)	

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 þ No ¨

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes þ 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 þ Accelerated filer ¨

Non-accelerated filer ¨ Smaller reporting company ¨

Emerging growth company ¨

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

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

The number of shares of outstanding common stock, par value $0.0001 per share, of the Registrant as of April 30, 2018 was 39,943,960.

Explanatory Note

Retrophin, Inc. (the "Company") is filing this Amendment No. 1 (the "Amendment") to its Quarterly Report on Form 10-Q for the period ended March 31, 2018, as filed with the Securities and Exchange Commission on May 1, 2018 (the "Original Filing"), solely to correct a clerical error in Exhibits 32.1 and 32.2 attached to the Original Filing. Except as described above, no other changes have been made to the Original Filing. Accordingly, this Amendment does not reflect events occurring after the filing of the Original Filing or modify or update those disclosures affected by subsequent events. Information not affected by this Amendment remains unchanged and reflects the disclosures made at the time the Original Filing was filed. Therefore, this Amendment should be read in conjunction with any documents incorporated by reference therein and the Company's filings made with the Securities and Exchange Commission subsequent to the Original Filing.

RETROPHIN, INC.

Form 10-Q/A

For the Fiscal Quarter Ended March 31, 2018

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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 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, 2017 (the "2017 10-K"), and in this Amendment 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.

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PART I - FINANCIAL INFORMATION
Item 1. Financial Statements
RETROPHIN, INC. AND SUBSIDIARIES
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share amounts)

	March 31, 2018 (unaudited)	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$61,117	$99,394
Marketable securities	202,939	201,236
Accounts receivable, net	12,981	13,872
Inventory, net	5,142	5,351
Prepaid expenses and other current assets	2,011	3,112
Prepaid taxes	2,613	2,842
Total current assets	286,803	325,807
Property and equipment, net	3,042	3,230
Other assets	6,457	5,556
Investment-equity	15,000	—
Intangible assets, net	188,556	184,817
Goodwill	936	936
Total assets	$500,794	$520,346
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$9,423	$18,938
Accrued expenses	31,644	36,018
Guaranteed minimum royalty	2,000	2,000
Other current liabilities	3,958	3,902
Business combination-related contingent consideration	9,500	9,100
Derivative financial instruments, warrants	—	15,710
Total current liabilities	56,525	85,668
Convertible debt	45,238	45,077
Other non-current liabilities	4,617	2,472
Guaranteed minimum royalty, less current portion	12,939	13,095
Business combination-related contingent consideration, less current portion	82,000	80,900
Total liabilities	201,319	227,212
Stockholders' Equity:		
Preferred stock $0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2018 and December 31, 2017	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 39,873,285 and 39,373,745 issued and outstanding as of March 31, 2018 and December 31, 2017, respectively	4	4
Additional paid-in capital	486,717	471,800
Accumulated deficit	(185,717)	(177,655)
Accumulated other comprehensive loss	(1,529)	(1,015)
Total stockholders' equity	299,475	293,134
Total liabilities and stockholders' equity	$500,794	$520,346

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

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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,	
	2018	2017
Net product sales	$38,432	$33,620
Operating expenses:		
Cost of goods sold	1,613	709
Research and development	24,636	20,860
Selling, general and administrative	26,468	23,115
Change in fair value of contingent consideration	3,627	3,344
Total operating expenses	56,344	48,028
Operating loss	(17,912)	(14,408)
Other income (expenses), net:		
Other income, net	121	126
Interest expense, net	(358)	(132)
Change in fair value of derivative instruments	—	1,260
Total other income (expense), net	(237)	1,254
Loss before provision for income taxes	(18,149)	(13,154)
Income tax benefit (expense)	(229)	2,064
Net loss	$(18,378)	$(11,090)
Net loss per common share:		
Basic	$(0.46)	$(0.29)
Diluted	$(0.46)	$(0.32)
Weighted average common shares outstanding:		
Basic	39,657,418	38,045,317
Diluted	39,657,418	39,158,922
Comprehensive loss:		
Net loss	$(18,378)	$(11,090)
Foreign currency translation	22	(76)
Unrealized gain (loss) on marketable securities	(536)	146
Comprehensive loss	$(18,892)	$(11,020)

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

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

	For the Three Months Ended March 31,	
	2018	2017
Cash Flows From Operating Activities:		
Net loss	$(18,378)	$(11,090)
Adjustments to reconcile net income to net cash provided by operating activities:		
Depreciation and amortization	4,348	4,284
Deferred income tax benefit	—	(2,064)
Interest income from notes receivable	—	(325)
Non-cash interest expense	413	376
Amortization of premiums on marketable securities	356	287
Share based compensation	4,659	7,093
Change in fair value of liability classified warrants	—	(1,260)
Change in fair value of contingent consideration	3,627	3,344
Payments related to change in fair value of contingent consideration	(4,245)	(914)
Inventory Reserve	816	25
Other	75	53
Changes in operating assets and liabilities, net of business acquisitions:		
Accounts receivable	886	3,076
Inventory	(593)	(574)
Other current and non-current operating assets	421	(946)
Accounts payable and accrued expenses	(9,330)	(4,626)
Other current and non-current operating liabilities	2,211	(146)
Net cash used in operating activities	(14,734)	(3,407)
Cash Flows From Investing Activities:		
Purchase of fixed assets	(39)	(838)
Cash paid for intangible assets	(8,217)	(3,477)
Proceeds from the sale/maturity of marketable securities	26,924	8,440
Purchase of marketable securities	(29,519)	—
Cash paid for investments - equity	(10,000)	—
Net cash provided by (used in) investing activities	(20,851)	4,125
Cash Flows From Financing Activities:		
Payment of acquisition-related contingent consideration	(7,066)	(1,068)
Payment of guaranteed minimum royalty	(500)	(500)
Payment of other liability	—	(250)
Proceeds from exercise of warrants	608	—
Proceeds from exercise of stock options	4,256	1,964
Net cash provided by (used in) financing activities	(2,702)	146
Effect of exchange rate changes on cash	10	4
Net change in cash	(38,277)	868
Cash, beginning of year	99,394	41,002
Cash, end of period	$61,117	$41,870

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

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RETROPHIN, INC. AND SUBSIDIARIES
NOTES TO THE UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATMENTS
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 our direct and indirect subsidiaries. Retrophin is a fully integrated biopharmaceutical company headquartered in San Diego, California focused on the development, acquisition and commercialization of therapies for the treatment of 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. Consequences of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. 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.
Sparsentan (RE-021) is an investigational product candidate which acts as both a potent angiotensin receptor blocker (“ARB”), as well as a selective endothelin receptor antagonist (“ERA”), with in vitro selectivity toward endothelin receptor type A. The Company secured a license to sparsentan from Ligand Pharmaceuticals, Inc. and Bristol-Myers Squibb Company (who referred to it as DARA). The Company is developing sparsentan as a treatment for:
Focal segmental glomerulosclerosis ("FSGS"), which is a leading cause of end-stage renal disease and nephrotic syndrome (“NS”). There are no U.S. Food and Drug Administration ("FDA") approved pharmacologic treatments for FSGS and off-label resources are limited to ACE/ARBs, steroids, and immunosuppressant agents, which are effective in only a subset of patients.
Immunoglobulin A nephropathy ("IgAN"), which is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. There is no FDA approved treatment for IgAN.
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"). 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.
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.
The Company is party to a three-way Cooperative Research and Development Agreement ("CRADA") with the National Institutes of Health’s National Center for Advancing Translational Sciences and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for N-glycanase deficiency ("NGLY1").
NGLY1 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 has been 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.
Liquid ursodeoxycholic acid ("L-UDCA") is a liquid formulation of ursodeoxycholic acid being developed for the treatment of a rare liver disease called primary biliary cholangitis ("PBC"). The Company obtained the rights to L-UDCA in 2016 with the intention of making L-UDCA commercially available to the subset of PBC patients who have difficulty swallowing.
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 also 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 (approved and marketed in Europe for select indications as Kolbam) 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.

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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 2017 10-K filed with the SEC on February 27, 2018. 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, 2017 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

Effective January 1, 2018, the Company adopted Accounting Standards Codification (" ASC"), Topic 606, Revenue from Contracts with Customers, using the full retrospective transition method. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under Topic 606, an entity 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 the consideration it is entitled to in exchange for the goods or services it transfers to the customer. See Note 3 for further discussion.

Adoption of New Accounting Standards

In May 2014, the Financial Accounting Standard Board ("FASB") issued ASU No. 2014-09, Revenue from Contracts with Customers. Under the new standard, revenue is recognized at the time a good or service is transferred to a customer for the amount of consideration for which the entity expects to be entitled for that specific good or service. Entities may use a full retrospective approach or report the cumulative effect as of the date of adoption. The Company adopted the new standard on January 1, 2018 using the full retrospective approach and there was no impact on the timing or recognition of revenue because its only revenue source is product sales and because no variable consideration exists. The new standard also requires enhanced disclosures about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts. See Note 3 for further discussion.

In July 2017, the FASB issued ASU No. 2017-11, Earnings Per Share (Topic 260); Distinguishing Liabilities from Equity (Topic 480); Derivatives and Hedging (Topic 815): (Part I) Accounting for Certain Financial Instruments with Down Round Features. These amendments simplify the accounting for certain financial instruments with down round features. The amendments require companies to disregard the down round feature when assessing whether the instrument is indexed to its own stock, for purposes of determining liability or equity classification. See Note 6 for further discussion.

In January 2017, the FASB issued ASU 2017-01, Business Combinations (Topic 805): Clarifying the Definition of a Business. The new guidance dictates that, when substantially all of the fair value of the gross assets acquired (or

disposed of) is concentrated in a single identifiable asset or a group of similar identifiable assets, it should be treated as an acquisition or disposal of an asset. The guidance was adopted as of January 1, 2018.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies. Unless otherwise discussed, 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 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. A modified retrospective transition approach is required for lessees for capital and operating leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, with certain practical expedients available. The Company is in the process of evaluating the impact of this guidance on its consolidated financial statements and related disclosures; however, based on the Company's current operating leases, it is expected to have a material impact on the company's consolidated balance sheet by increasing assets and liabilities.

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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, 2018, the Company holds $202.9 million in available for sale debt securities that are affected by this ASU. If adopted as of March 31, 2018, this would not have a material impact on the company's financial statements.

In February 2018, the FASB issued ASU 2018-02, Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income. The new guidance addresses a specific consequence of the newly enacted federal income tax law (the "Tax Act"). This accounting update allows a reclassification from accumulated other comprehensive income to retained earnings for stranded tax effects resulting from the Tax Act. The amendments eliminate the stranded tax effects that were created as a result of the reduction of historical U.S. federal corporate income tax rates to the newly enacted U.S. federal corporate income tax rates. The accounting update is effective January 1, 2019, with early adoption permitted, and is to be applied either in the period of adoption or retrospectively to each period in which the effect of the change in the U.S. federal corporate income tax rate in the Tax Act is recognized. The Company is currently evaluating the potential effect of the guidance on its consolidated 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 is 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 Company had the following net product revenues (in thousands):

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	For the three months ended:	
	March 31, 2018	March 31, 2017
Bile acid products	$18,508	$15,736
Thiola	19,924	17,884
Total net product revenue	$38,432	$33,620

NOTE 4. MARKETABLE SECURITIES

The Company's marketable securities as of March 31, 2018 and December 31, 2017 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, 2018, investment activity for the Company included $26.9 million in maturities and $29.7 million in purchases, all relating to debt based marketable securities.

Marketable securities consisted of the following (in thousands):

	March 31, 2018	December 31, 2017
Marketable Securities:		
Commercial paper	$10,339	$6,897
Corporate debt securities	162,648	164,297
Securities of government sponsored entities	29,952	30,042
Total marketable securities:	$202,939	$201,236

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

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Losses	Aggregate Estimated Fair Value
Marketable securities:				
Commercial paper	Less than 1	$10,384	$(45)	$10,339
Corporate debt securities	Less than 1	88,525	(439)	88,086
Securities of government-sponsored entities	Less than 1	30,019	(67)	29,952
Total maturity less than 1 year		128,928	(551)	128,377
Corporate debt securities	1 to 2	75,276	(714)	74,562
Total maturity 1 to 2 years		75,276	(714)	74,562
Total available-for-sale securities		$204,204	$(1,265)	$202,939

The following is a summary of short-term marketable securities classified as available-for-sale as of December 31, 2017 (in thousands):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Losses	Aggregate Estimated Fair Value
Marketable securities:				
Commercial paper	Less than 1	$6,911	$(14)	$6,897
Corporate debt securities	Less than 1	86,531	(198)	86,333
Securities of government-sponsored entities	Less than 1	30,132	(90)	30,042
Total maturity less than 1 year		123,574	(302)	123,272

Corporate debt securities	1 to 2	78,388	(424)	77,964
Total maturity 1 to 2 years		78,388	(424)	77,964
Total available-for-sale securities		$201,962	$ (726)	$201,236

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.

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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, 2018 and December 31, 2017, 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 Pharmaceuticals Inc. ("Censa"), which became effective in January 2018. The Company has 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 has 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. In exchange for the Option, the Company paid $10 million, and an additional $5 million upon Censa's completion of a specified development milestone set forth in the Option Agreement.

If the Company exercises the Option, the Company will acquire Censa for $65 million in upfront consideration, subject to certain adjustments, 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") 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.

As of March 31, 2018, the Company has paid $10.0 million as an upfront payment and $5.0 million in development funding to Censa. In addition, the Company accrued $5.0 million related to a development milestone payable to Censa within 45 days of achievement. The Company capitalized the upfront payment and accrued milestone, and expensed the development funding paid. The Company is treating the upfront payment and milestone, both of which are compensation for the purchase option, as a cost-method investment with a total carrying value of $15.0 million as March 31, 2018. The Company's maximum exposure to loss related to this VIE is limited to the carrying value of its investment.

NOTE 6. DERIVATIVE FINANCIAL INSTRUMENTS

Since 2013, the Company has issued five tranches of common stock purchase warrants to secure financing, remediate covenant violations and provide consideration for amendments with respect to a credit facility extinguished in 2015. Historically, the Company accounted for these instruments, which do not have fixed settlement provisions, as derivative instruments in accordance with FASB ASC 815-40, Derivative and Hedging – Contracts in Entity's Own Equity. This was due to an anti-dilution provision for the warrants that provides for a reduction to the exercise price if the Company issues equity or equity linked instruments in the future at an effective price per share less than the exercise price then in effect for the warrant ("down round provision"). As such, the warrants were re-measured at each balance sheet date based on estimated fair value. Changes in estimated fair value were recorded as non-cash adjustments within other income (expenses), net, in the Company's accompanying Condensed Consolidated Statements of Operations and Comprehensive Loss. The Company recorded a gain on the change in the estimated fair value of warrants of $1.3 million for the three months ended March 31, 2017.

As of January 1, 2018 the Company early adopted ASU 2017-11, which revised the guidance for instruments with down round provisions. As such the Company treats outstanding warrants as free-standing equity linked instruments that will be recorded to equity in the Consolidated Balance Sheet.

In accordance with the guidance presented in the ASU, the fair value of the derivative liability balance as of December 31, 2017 of $15.7 million was reclassified by means of a cumulative-effect adjustment to equity as of January 1, 2018.

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Impact of the adoption of ASU 2017-11 on equity (in thousands):

	Common stock	Additional paid in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity (deficit)
Balance–December 31, 2017	$ 4	$ 471,800	$ (1,015)	$ (177,655)	$ 293,134
Balance-March 31, 2018 before effect of ASU 17-11	4	481,323	(1,529)	(196,033)	283,765
Effect of ASU 17-11	—	5,394		10,316	15,710
Balance–March 31, 2018	$ 4	$ 486,717	$ (1,529)	$ (185,717)	$ 299,475

The Company calculated the fair value of the warrants using the Black-Scholes Model as of December 31, 2017, using the following assumptions:

	December 31, 2017
Fair value of common stock	$ 21.07
Remaining life of the warrants (in years)	0.1 - 2.0 years
Risk-free interest rate*	1.39 - 1.89%
Expected volatility**	33 - 43%
Dividend yield	— %

*The risk-free interest rate is based on the U.S. Treasury security rates for the remaining term of the warrants at the measurement date.

**Expected volatility is based on an analysis of the Company's historical volatility.

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 derivative liabilities, the Company used the Black Scholes Model as of December 31, 2017. Based on the fair value hierarchy, the Company classified the derivative liability within Level 3. The Company adopted ASU 2017-11 as of January 1, 2018 and is no longer required to treat outstanding warrants as derivative liabilities measured at fair value. See Note 6 for further discussion.

In estimating the fair value of the Company's contingent consideration, the Company used the probability-based expected method for royalty payments based on projected revenues. Based on the fair value hierarchy, the Company classified contingent consideration within Level 3 because valuation inputs are based on projected revenues

discounted to a present value.

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, 2018, the estimated fair value of convertible debt was $63.4 million, considering factors such as market conditions, prepayment and make-whole provisions, variability in pricing from multiple lenders and the term of the debt.

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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, 2018 (in thousands):

	As of March 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	$61,117	$61,117	$—	$ —
Marketable securities, available-for-sale	202,939	—	202,939	—
Total	$264,056	$61,117	$ 202,939	$ —
Liabilities:				
Business combination-related contingent consideration	$91,500	$—	$—	$ 91,500
Total	$91,500	$—	$—	$ 91,500

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, 2017 (in thousands):

	As of December 31, 2017			
	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	$99,394	$92,726	$ 6,668	$ —
Marketable securities, available-for-sale	201,236	—	201,236	—
Total	$300,630	$92,726	$ 207,904	$ —
Liabilities:				
Derivative liability related to warrants	$15,710	$—	$—	$ 15,710
Business combination-related contingent consideration	90,000	—	—	90,000
Total	$105,710	$—	$—	$ 105,710

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, 2018 (in thousands):

	Fair Value Measurements of Acquisition-Related Contingent Consideration (Level 3)
Balance at January 1, 2018	$ 90,000
Increase from revaluation of contingent consideration	3,627
Contractual payments included in accrued liabilities at March 31, 2018	(2,127)
Contractual payments	—
Balance at March 31, 2018	$ 91,500

The fair value of contingent consideration liabilities was determined by applying a form of the income approach (a level 3 input), based upon the probability-weighted projected payment amounts discounted to present value at a rate appropriate for the risk of achieving the performance targets. The key assumptions included in the calculations were

the earn-out period payment probabilities, 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, 2018, the Company incurred charges of $3.6 million in operating expenses on the Condensed Consolidated Statements of Operations and Comprehensive Loss for the revaluation 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 changes in the estimated timing of payments. During the three months ended March 31, 2017, the Company incurred charges of $3.3 million in operating expenses on the Condensed Consolidated Statement of Operations and Comprehensive Loss for the revaluation of the contingent consideration liabilities. For the three months ended March 31, 2017, $1.4 million, $1.1 million, and $0.8 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 changes in the estimated timing of payments.

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NOTE 8. INTANGIBLE ASSETS

As of March 31, 2018, the net book value of amortizable intangible assets was approximately $188.6 million. The following table sets forth amortizable intangible assets as of March 31, 2018 and December 31, 2017 (in thousands):

	March 31, 2018	December 31, 2017
Finite-lived intangible assets	$243,865	$235,863
Less: accumulated amortization	(55,309)	(51,046)
Net carrying value	$188,556	$184,817

The following table summarizes amortization expense for the three months ended March 31, 2018 and 2017 (in thousands):

	Three Months Ended March 31,	
	2018	2017
Research and development	$103	$81
Selling, general and administrative	4,096	4,090
Total amortization expense	$4,199	$4,171

During the three months ended March 31, 2018, the Company made a development payment to Ligand Pharmaceuticals for $4.6 million relating to sparsentan which was recorded as an increase to the Ligand license intangible asset.

NOTE 9. CONVERTIBLE NOTES PAYABLE

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 senior convertible notes due in 2019 (the “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 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 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. At March 31, 2018 and December 31, 2017, the aggregate carrying value of the Notes was $45.2 million and $45.1 million, respectively.

NOTE 10. ACCRUED EXPENSES

Accrued expenses at March 31, 2018 and December 31, 2017 consisted of the following (in thousands):

	March 31, 2018	December 31, 2017
Government rebates payable	$6,372	$ 5,883
Compensation related costs	5,693	7,749
Accrued royalties and contingent consideration	5,870	6,429
Research and development	9,553	6,989
Selling, general and administrative	2,058	3,896
Restructuring expenses	42	3,549
Miscellaneous accrued	2,056	1,523
Total accrued expenses	$31,644	$ 36,018

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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 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,					
	2018			2017		
	Shares	Net Loss	EPS	Shares	Net Loss	EPS
Basic loss per share	39,657,418	$(18,378)	$(0.46)	38,045,317	$(11,090)	$(0.29)
Dilutive shares related to warrants	—	—		1,113,605	—	
Change in fair value of derivative instruments	—	—		—	(1,260)	
Dilutive loss per share	39,657,418	$(18,378)	$(0.46)	39,158,922	$(12,350)	$(0.32)

The following shares were excluded because they were anti-dilutive (in thousands):

	Three Months Ended March 31,	
	2018	2017
Restricted stock units	139	332
Convertible debt	2,642	2,642
Options	7,025	6,405
Warrants	633	—
Total anti-dilutive shares	10,439	9,379

NOTE 12. COMMITMENTS AND CONTINGENCIES

Leases and Sublease Agreements

Facilities	Annual Base Rent	Lease Expiration	Comments
Occupied Locations			
Corporate Headquarters San Diego, CA	$2.1 million	July 2024	
Vacated Locations			
New York, NY	$0.5 million	November 2018	Sublet through expiration

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. In connection with these proceedings, Mr. Shkreli sought advancement of his legal fees from the Company. The Company disputed its obligation to pay these amounts in full, and in November 2016, the Company and Mr. Shkreli entered into a binding settlement arrangement under which the Company advanced $2.8 million in legal fees to Mr. Shkreli's counsel. The Company also advanced an additional $2 million in legal fees once the matter proceeded to trial. In December 2017, the Company agreed to advance Mr. Shkreli $625,000 in full satisfaction of its obligation to

advance fees to Mr. Shkreli in connection with his appeal of his conviction. The Company has been reimbursed by its directors' and officers' insurance carriers for $3.3 million of the legal fees the Company advanced in connection with the pre-trial and trial proceedings. 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

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final amount of the reimbursement from the insurance carriers is not currently estimable. In addition, a portion of the 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. The federal Court granted a stay of the federal lawsuit pending a determination by the arbitration panel whether the Company's counterclaims would be litigated in the arbitration, as the Company is seeking. In April 2016, the arbitration panel granted the parties' request for a stay of the proceedings pending settlement discussions. 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. Pursuant to the November 2016 binding term sheet, the significant majority of the existing legal fees related to these proceedings that Mr. Shkreli claimed should be advanced were offset and satisfied by a $2.025 million judgment against Mr. Shkreli in a different case, and the Company paid $0.4 million in legal fees to Mr. Shkreli's counsel. The legal fees the Company has advanced 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. The Company will also be subject to additional obligations when the litigation resumes, as well as advancement obligations in the interim.

For the three months ended March 31, 2018 and 2017, the Company recorded no expenses under the settlement agreement. For the three months ended March 31, 2017, the Company paid $1.0 million under the settlement. No reimbursements from the Company's directors' and officers' insurance carriers were received during the three months ended March 31, 2018 and 2017. Of the $3.3 million reimbursed by the insurance carriers, $2.6 million is recorded as a liability on the Consolidated Balance Sheet pending the outcome of Mr. Shkreli's appeal.

From time to time the Company is involved in legal proceedings arising in the ordinary course of business. The Company believes there is no litigation pending 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 and Performance Based Restricted Stock Units

The following table summarizes the Company's restricted stock activity during the three months ended March 31, 2018:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested December 31, 2017	345,332	$ 20.51
Granted	18,061	22.18
Vested	(11,377)	18.33
Forfeited/canceled	(12,666)	22.84
Unvested March 31, 2018	339,350	$ 20.58

At March 31, 2018, unamortized stock compensation for restricted stock units was $2.1 million, with a weighted-average recognition period of 1.2 years.

Performance Based Restricted Stock Units

The Company did not grant any performance based restricted stock units during the three months ended March 31, 2018.

Stock Options

The following table summarizes stock option activity during the three months ended March 31, 2018:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2017	7,153,668	$ 17.16	6.95	$ 39,010
Granted	136,500	24.08		
Exercised	(319,551)	13.32		
Forfeited/canceled	(198,002)	25.35		
Outstanding at March 31, 2018	6,772,615	$ 17.25	7.02	$ 43,054

At March 31, 2018, unamortized stock compensation for stock options was $24.7 million, with a weighted-average recognition period of 2.6 years.

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At March 31, 2018, outstanding options to purchase 4.5 million shares of common stock were exercisable with a weighted-average exercise price per share of $16.16.

Share Based Compensation

The following table sets forth total non-cash stock-based compensation for the three months ended March 31, 2018 and 2017 (in thousands):

	Three Months Ended March 31,	
	2018	2017
Research and development	$1,407	$2,656
Selling, general & administrative	3,202	4,437
Total	$4,609	$7,093

Exercise of Warrants

During the three months ended March 31, 2018, the Company issued 168,612 shares of common stock upon the exercise of outstanding warrants for cash received by the Company in the amount of $0.6 million. See Note 6 for changes in warrant accounting.

At March 31, 2018 and December 31, 2017, warrants to purchase 990,810 and 1,159,424 shares of common stock, respectively, were outstanding.

NOTE 14. INCOME TAXES

The following table summarizes our effective tax rate and income tax benefit (expense) for the three months ended March 31, 2018 and 2017 (dollars in millions):

	Three Months Ended March 31,	
	2018	2017
Effective tax rate	(1.3)%	15.7%
Income tax benefit (expense)	$(0.2)	$2.1

For the three months ended March 31, 2018 and 2017, we recognized an income tax expense of $0.2 million and an income tax benefit of $2.1 million, respectively, representing an effective tax rate of (1.3)% and 15.7%, respectively. 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, 2018, when compared to the same period in 2017, the change in the tax benefit and decrease in the effective income tax rate was primarily attributable to the increase of the U.S. federal valuation allowance in 2018.

At March 31, 2018, 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, 2018.

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act. The Tax Legislation reduces the US federal corporate tax rate from 35% to 21%, as well as making several other significant changes to the tax law, effective January 1, 2018. Pursuant to the Securities and Exchange Commission Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act (SAB 118), given the amount and complexity of the changes in tax law resulting from the Tax Legislation, the Company has not finalized the accounting for the income tax effects of the Tax Legislation. This includes the provisional amounts recorded related to the re-measurement of the deferred taxes and the change to our valuation allowance. The impact of the Tax Legislation may differ from this estimate during the one-year measurement period due to, among other things, further refinement of the Company's calculation, changes in interpretations and assumptions the Company has made, guidance that may be issued and actions the Company may take as a result of the Tax Legislation.

At March 31, 2018, the Company is still analyzing certain aspects of the Tax Legislation and refining its calculations, which could potentially affect the analysis of the Company’s deferred tax assets and liabilities and its valuation

allowance. Any subsequent adjustment is not expected to be material to the Company's financial position or results of operations.

NOTE 15. INVENTORY

Inventory, net of reserves, consisted of the following at March 31, 2018 and December 31, 2017 (in thousands):

	March 31, 2018	December 31, 2017
Raw materials	$ 3,743	$ 3,435
Finished goods	1,399	1,916
Total inventory	$ 5,142	$ 5,351

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The inventory reserve was $1.3 million and $0.7 million at March 31, 2018 and December 31, 2017, respectively.

NOTE 16. ACCOUNTS RECEIVABLES

Accounts receivable, net of reserves for prompt pay discounts and doubtful accounts, was $13.0 million and $13.9 million at March 31, 2018 and December 31, 2017, respectively. The total reserves for both periods were immaterial.

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 Amendment and the audited financial statements and notes thereto as of and for the year ended December 31, 2017 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, 2017, filed with the Securities and Exchange Commission (SEC) on February 27, 2018. 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 Amendment 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.

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Our Product Candidates and Products on the Market

**Acquired rights in 2016; activities underway with the intention of making the liquid formulation commercially available in the United States.

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. Consequences 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 an IND, completed a Phase I clinical trial and obtained both orphan drug and fast track designations in the United States. Additionally, we received orphan drug designation in the European Union and reached an agreement with the U.S. Food and Drug administration ("FDA") under the Special Protocol Assessment (SPA) process for a Phase 3 clinical trial for PKAN. In July 2017, the first patient was dosed in our Phase 3 FORT (FOsmetpantotenate Replacement Therapy) study and enrollment continues.

Sparsentan

Sparsentan is an investigational product candidate which acts as both a potent angiotensin receptor blocker (“ARB”), as well as a 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 Company (who referred to it as DARA). We are developing sparsentan as a treatment for:

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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 resources 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 received positive data from our Phase 2 DUET study of sparsentan for the treatment of FSGS. In April 2018, we announced the initiation of the Phase 3 DUPLEX Study of sparsentan in FSGS. 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 Subpart H accelerated approval of sparsentan 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.

Immunoglobulin A nephropathy ("IgAN"), which is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of more than 100,000 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. Based on recent interactions we have had with the FDA and EMA, we are planning to initiate a single Phase 3 clinical trial designed to serve as the basis for an NDA filing for sparsentan for the treatment of IgAN. We are currently working to harmonize the protocol design for this Phase 3 study by incorporating the feedback to guide our clinical activity. We expect to initiate this study during the fourth quarter of 2018.

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, on January 8, 2018, we paid Censa $10 million, and are required to pay Censa 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 is currently being evaluated in single and multiple ascending dose studies and a Phase 2 proof of concept study in PKU is expected to commence in mid-2018.

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.

If we exercise the Option, pursuant to the terms of the Merger Agreement, we will acquire Censa for $65 million in upfront consideration, subject to certain adjustments, paid as a combination of 20% in cash and 80% in shares of our

common stock, valued at a fixed price of $21.40 per share; provided, however, that Censa may elect on behalf of its equityholders to receive the upfront consideration in 100% cash if the average price per share of our common stock for the ten trading days ending on the date we provide a notice of interest to exercise the Option is less than $19.26. In addition to the upfront consideration, if we exercise the Option and acquire Censa, we would be required to make further cash payments to Censa's equityholders of up to an aggregate of $25 million if the CNSA-001 program achieves specified development and commercial milestones.

NGLY1 Deficiency Discovery Efforts

N-glycanase deficiency, or 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.

We are party to a three-way Cooperative Research and Development agreement with the National Institutes of Health's National Center for Advancing Translational Sciences and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency.

Liquid Ursodeoxycholic Acid

Liquid ursodeoxycholic acid ("L-UDCA") is a liquid formulation of ursodeoxycholic acid being developed for the treatment of a rare liver disease called primary biliary cholangitis ("PBC"). We obtained L-UDCA in 2016 with the intention of making L-UDCA commercially available to the subset of PBC patients who have difficulty swallowing. There are no liquid formulations of ursodeoxycholic acid currently approved by the FDA.

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We sell the following three products:

Chenodal® (chenodiol)

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 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 ANDA for Chenodal was approved by the FDA for the treatment of gallstones; Chenodal is manufactured for Manchester Pharmaceuticals LLC ("Manchester") under this ANDA. Manchester subsequently obtained orphan drug designation for Chenodal for the treatment of CTX, a rare autosomal recessive lipid storage disease, in 2010. Manchester was acquired by us 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 United States 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 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)

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. The estimated incidence of bile acid synthesis disorders due to single enzyme defects is 1 to 9 per million live births.

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. We are currently developing a new, more patient-friendly, formulation of Thiola for which an NDA is expected in 2018.

Results of Operations

Results of operations for the three months ended March 31, 2018 compared to the three months ended March 31, 2017.

Net Product Sales:

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

	Three Months Ended March 31,		
	2018	2017	Change
Net product revenues by product:			
Bile acid products	$18,508	$15,736	$2,772
Thiola	19,924	17,884	2,040
Total net product revenues	$38,432	$33,620	$4,812

The increase in sales for the three months ended March 31, 2018 over the prior year is due to increased patient counts for Chenodal, Cholbam and Thiola.

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Operating Expenses:

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

	Three Months Ended March 31,		
	2018	2017	Change
Cost of goods sold	$1,613	$709	$904
Research and development	24,636	20,860	3,776
Selling, general and administrative	26,468	23,115	3,353
Change in fair value of contingent consideration	3,627	3,344	283
	$56,344	$48,028	$8,316

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, 2018 as compared to the three months ended March 31, 2017, the Company increased its research and development expenses by $3.8 million, which is due to increased clinical trial expenses.

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, 2018 as compared to the same period ended March 31, 2017, the Company increased its selling, general and administrative expenses by $3.4 million, which reflects the increase in the Company's activities to support corporate initiatives.

Change in the valuation of contingent consideration

For the three months ended March 31, 2018 as compared to the three months ended March 31, 2017, the change in fair value of contingent consideration is due to changes in the timing of payments and discount factors.

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

	Three Months Ended March 31,		
	2018	2017	Change
Chenodal	$801	$1,440	$(639)
Cholbam	1,826	1,104	722
L-UDCA	1,000	800	200
	$3,627	$3,344	$283

Other Income/Expenses:

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

	Three Months Ended March 31,		
	2018	2017	Change
Other income, net	$121	$126	$(5)
Interest expense, net	(358)	(132)	(226)
Change in fair value of derivative instruments	—	1,260	(1,260)
	$(237)	$1,254	$(1,491)

The changes in the Company's other income (expenses) for the three months ended March 31, 2018 and 2017 of $1.5 million is primarily due to changes in the accounting method for the fair value of derivative instruments related to warrants. See Note 6 of the Consolidated Financial Statements for further discussion.

Income Tax Benefit:

The Company recorded a tax expense of $0.2 million for the three months ended March 31, 2018 related to state franchise taxes and installment sale interest.

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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, 2018 and December 31, 2017 (in thousands):

	March 31, 2018	December 31, 2017
Cash & Cash Equivalents	$61,117	$ 99,394
Marketable securities	202,939	201,236
Accumulated Deficit	(185,717)	(177,655)
Stockholders' Equity	299,475	293,134
Net Working Capital*	$230,278	$ 240,139
Net Working Capital Ratio**	5.07	3.80

* Current assets less current liabilities.

**Current assets divided by current liabilities.

Convertible Notes Payable

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 senior convertible notes due in 2019 (the "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 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 Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with the terms. The aggregate carrying value of the Notes was $45.2 million and $45.1 million at March 31, 2018 and December 31, 2017, respectively.

Cash Flows from Operating Activities

Cash used in operating activities was $14.7 million and $3.4 million for the three months ended March 31, 2018 and 2017, respectively. After disregarding non-cash changes such as derivative liability fluctuations, share based compensation, contingent consideration charges and deferred tax asset reserves, the increase of $11.3 million was primarily due to payments to Censa for development funding and changes in operating liabilities.

Cash Flows from Investing Activities

Cash used in investing activities for the three months ended March 31, 2018 was $20.9 million, compared to $4.1 million cash provided by investing activities for the three months ended March 31, 2017. The change was primarily due to cash paid for investments in equities, intangibles, and changes in marketable securities maturities and related reinvestment.

Cash Flows from Financing Activities

Cash used in financing activities for the three months ended March 31, 2018 was $2.7 million compared to cash provided of $0.1 million for the three months ended March 31, 2017. The decrease was due to the payment of a sales milestone for a bile acid product, partially offset by higher proceeds from stock option and warrant exercises in the first quarter of 2018.

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;

- 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;

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- 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 Option Agreement with Censa Pharmaceuticals Inc., 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 $1.6 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, 2018, 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

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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 Amendment.

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, 2017, 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 Amendment 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 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 certain 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 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.

If physicians, patients and third-party payers do not accept our products, we may be unable to generate significant revenues.

Our drugs may not gain or maintain market acceptance among physicians and patients. Effectively marketing our products and any of our drug candidates, if approved, requires substantial efforts, both prior to launch and after approval. Physicians may elect not to prescribe our drugs, and patients may elect not to request or take them, for a variety of reasons including:

- lower demonstrated efficacy, safety and/or tolerability compared to other drugs;
- prevalence and severity of adverse side-effects;
- lack of cost-effectiveness;
- lack of coverage and adequate reimbursement availability from third-party payers;
- a decision to wait for the approval of other therapies in development that have significant perceived advantages over our drug;
- convenience and ease of administration;
- other potential advantages of alternative treatment methods; and
- ineffective marketing and/or distribution support.

If our drugs fail to achieve or maintain market acceptance, we will not be able to generate significant revenues.

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, CNSA-001 and L-UDCA, or any other product candidate that we develop, restrict or regulate

post-approval activities and affect our ability to profitably sell fosmetpantotenate, sparsentan, CNSA-001 and L-UDCA 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

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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 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. Additional competitors could enter the market, including with generic versions of our products, and sales of our affected products may decline materially.

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

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, 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.

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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.

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.

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. 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. We cannot assure that any current or future clinical trials of fosmetpantotenate and/or sparsentan 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.

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.

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 2018, we initiated a single Phase 3 clinical trial designed to serve as the basis for an NDA filing for sparsentan for the treatment of FSGS. Although we received feedback from the FDA at an End of Phase 2 meeting during which the FDA communicated that it was open to accepting a substantial treatment effect on proteinuria in this trial 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 such analyses will be sufficient to serve as the basis for an NDA filing, including an NDA under Subpart H or accelerated approval. In addition, our statistical modeling that supports proceeding 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 Phase 3 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 under Subpart H, there can be no assurance that the post-marketing confirmatory data will support full approval of sparsentan as a treatment for FSGS.

We are also pursuing the development of sparsentan as a treatment for IgAN. Based on our recent interactions with the FDA and EMA, we are planning to initiate 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. We are currently working to harmonize the protocol design for this Phase 3 study by incorporating the feedback to guide our clinical activity. Although we expect to initiate this study in the fourth quarter of 2018, there can be no assurance that the study will initiate on our expected timelines or proceed as planned.

Clinical trials can be lengthy, complex and extremely expensive processes with uncertain results. 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.

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:

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- 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.

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 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.

In addition, 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.

FDA approval for a product requires substantial or extensive preclinical and clinical data and supporting documentation. The approval process may take years and may involve on-going requirements as well as post marketing obligations. For example, we have certain post marketing requirements and commitments associated with Cholbam. FDA approval once obtained, may be withdrawn. 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. 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.

We face substantial risks related to the commercialization of our product candidates.

We have invested a significant portion of our efforts and financial resources in the development and acquisition of our most advanced product candidates, fosmetpantotenate, sparsentan, CNSA-001 and L-UDCA. Our ability to generate product revenue from these development stage compounds, which we do not expect will occur for at least the next several years, if ever, may depend heavily on the successful development and commercialization of these product candidates. The successful commercialization of our future product candidates will depend on several factors, including the following:

- obtaining supplies of fosmetpantotenate, sparsentan, CNSA-001 and subsequent product candidates for completion of our clinical trials on a timely basis;

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- successful completion of pre-clinical and clinical studies;
- with respect to L-UDCA, our ability to complete the activities necessary to submit an NDA;
- obtaining marketing approvals from the FDA and similar regulatory authorities outside the United States;
- establishing commercial-scale manufacturing arrangements with third-party manufacturers whose manufacturing facilities are operated in compliance with cGMP regulations;
- launching commercial sales of the product, whether alone or in collaboration with others;
- acceptance of the product by patients, the medical community and third-party payers;
- reimbursement from medical, medicaid or private payers;
- competition from other companies;
- successful protection of our intellectual property rights from competing products in the United States and abroad; and
- a continued acceptable safety and efficacy profile of our product candidates following approval.

Furthermore, 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 $16 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.

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 may not be able to obtain orphan drug exclusivity for our product candidates. If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Although we have obtained orphan designation for fosmetpantotenate and sparsentan, there can be no assurance that there will be any benefits associated with such designation.

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 if we have orphan drug exclusivity, 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 candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose what had previously been 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 before our product candidate is approved, 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

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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 fosmetpantotenate, sparsentan, CNSA-001 and L-UDCA, 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 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 or 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 fosmetpantotenate, 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.

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. Fosmetpantotenate 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.

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 or seven years regulatory

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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. 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. 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 fosmetpantotenate and sparsentan for the treatment of 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 ("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

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reforms. The PPACA revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of Medicaid drug rebates to states. Further, the law imposes a significant annual fee on companies that manufacture or import certain branded prescription drug products. Some of the provisions of the PPACA have yet to be implemented, and there have been judicial and Congressional 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 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 Cuts and Jobs Act of 2017 includes a provision repealing, 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”. Additionally, 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. Further, 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”. It is likely the PPACA, or a replacement of it under the current administration, will continue to put pressure on pharmaceutical pricing. We continue to evaluate the potential impact of the PPACA and its possible repeal or replacement on our business.

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 recent changes in the Medicare program and increasing emphasis on managed care to continue to put pressure on pharmaceutical product pricing.

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 proposed 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. While any proposed measures will 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.

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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.

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,

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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

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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;
- complete requirements necessary for an NDA filing of L-UDCA and 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 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, complete requirements for filings of L-UDCA, 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.

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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. For the twelve months ended December 31, 2017, we generated a positive cash flow from operations; however, 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;
- 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.

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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;
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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.

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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, Centers for Medicare and Medicaid Services, Department of Justice, the Federal Trade Commission, the Office of Inspector General of the U.S. Department of Health and Human Services 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. 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. 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.

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Many states also have statutes or regulations similar to the federal Anti-Kickback Statute and False Claims Act and civil monetary penalty laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, which apply regardless of the payer. In addition, several states require pharmaceutical companies to implement compliance programs or marketing codes as does the U.S. Department of Health and Human Services 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. While it is too early to predict the full effect these changes will have on our business, 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 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.

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.

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, which was officially adopted in April 2016 and will be applicable and enforced in May 2018, will introduce 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.

Failure to comply with U.S. and international data protection laws and regulations could result in government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, patients about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws,

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or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

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, the 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. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. 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. Several states now require pharmaceutical companies to report their expenses relating to the marketing and promotion of pharmaceutical products in those states and to report gifts and payments to certain individual health care providers in those states. Some of these states also prohibit certain marketing-related activities, including the provision of gifts, meals, and other items to certain health care providers.

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;

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- 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.

We face risks related to research and the ability to develop new drugs.

Our growth and survival depends on our ability to consistently discover, develop and commercialize new products and find new and improve on existing technology and platforms. As such, if we fail to make sufficient investments in research, be attentive to consumer needs or do not focus on the most advanced technology, our current and future products could be surpassed by more effective or advanced products of other companies.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law new legislation that significantly revises the Internal Revenue Code of 1986, as amended. The newly enacted federal income tax law, 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 earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, 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 new federal tax law 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 newly enacted

federal tax law. 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 this legislation 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 newly enacted federal income tax law, 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, 2017, we had federal net operating loss carryforwards of $5.2 million. These net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the newly enacted federal income tax law, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation

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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 net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. It is possible that we have experienced an ownership change limitation. 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. If an ownership change occurs and our ability to use our net operating loss carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

Risks Related to our Indebtedness and Investments

Our indebtedness could adversely affect our financial condition.

As of March 31, 2018, we had approximately $46.0 million of total debt outstanding, classified as long term. The total debt outstanding relates to a Note Purchase Agreement dated May 29, 2014 for the private placement of $46.0 million aggregate senior secured notes (the "Notes"). As a result of our indebtedness, a portion of our cash flow will be required to pay interest and principal on the 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 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 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.

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

If an event of default under the Notes occurs, the principal amount of the 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 Note;
- 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 Notes, unless cured or waived, may have a material adverse effect on our results of operations.

The Notes are structurally subordinated to all obligations of our subsidiaries.

The Notes are our obligations and are structurally subordinated to all indebtedness and other obligations, including trade payables, of our subsidiaries. The effect of this structural subordination is that, in the event of a bankruptcy, liquidation, dissolution, reorganization or similar proceeding involving a subsidiary which is not a guarantor of the Notes, the assets of the affected entity could not be used to pay noteholders until after all other claims against that subsidiary, including trade payables, have been fully paid.

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

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Certain provisions of the 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 Notes will have the right, at their option, to require us to repurchase all of their 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 Notes.

To the extent we issue shares of common stock upon conversion of the Notes, the conversion of some or all of the 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 Notes may encourage short selling by market participants because the conversion of the 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.

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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 Warrant Certificate, dated June 30, 2014, issued to the Lenders under the Credit Agreement (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on July 7, 2014).
4.2	Form of Common Stock Purchase Warrant, dated August 15, 2013, issued to the purchasers of securities in the private placement of the Company closed on August 15, 2013 (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on August 20, 2013).
4.3	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).
4.4	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).
4.5	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).
10.1	Form of Indemnity Agreement (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018)
10.2+	Amendment No. 5 to Sublicense Agreement dated as of March 20, 2018, between the Company and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018).
10.3†	Employment Agreement, dated February 13, 2017, and Amendment to Employment Agreement, dated April 11, 2017, by and between the Company and William Rote. (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018).
10.4†	Employment Agreement, dated February 6, 2017, and Amendment to Employment Agreement, dated April 11, 2017, by and between the Company and Elizabeth E. Reed (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018).
10.5†	2018 Retrophin, Inc. Executive Officer Annual Bonus Plan (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on March 9, 2018).
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

+ We have received confidential treatment of certain portions of this agreement, which have been omitted and filed separately with the SEC pursuant to Rule 406 under the Securities Act of 1933, as amended, or Rule 24b-2 of the Securities Exchange Act of 1934, as amended.

† Indicates management contract or compensatory plan.

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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: July 5, 2018 RETROPHIN, INC.

By: /s/ Stephen Aselage
Name: Stephen Aselage
Title: Chief Executive Officer

By: /s/ Laura Clague
Name: Laura Clague
Title: Chief Financial Officer