

Retrophin, Inc.  
Form 10-K  
February 26, 2016

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

FORM 10-K

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

For the Fiscal Year Ended December 31, 2015

Transition Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

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.)

12255 El Camino Real, Suite 250, San Diego, CA

92130

(Address of Principal Executive Offices)

(Zip code)

760-260-8600

(Registrant's telephone number, including area code)

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

Title of each class

Name of exchange on which registered

Common Stock, par value \$0.0001 per share

The NASDAQ Global Market

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act.  Yes  No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act.  Yes  No

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days.  Yes  No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files).

Yes  No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act):

Large Accelerated Filer

Accelerated Filer

Non-Accelerated Filer

Smaller Reporting Company

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).

Yes  No

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State the aggregate market value of the voting and non-voting common equity held by non-affiliates computed by reference to the price at which the common equity was last sold, or the average bid and asked price of such common equity, as of the last business day of the registrant's most recently completed second fiscal quarter. \$1,146,903,448. The number of shares of outstanding common stock, par value \$0.0001 per share, of the Registrant as of February 24, 2016 was 36,508,852.

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CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

Certain information contained in this Annual Report on Form 10-K of Retrophin, Inc., a Delaware corporation (the “Company”) include forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The statements herein which are not historical reflect our current expectations and projections about the Company’s future results, performance, liquidity, financial condition, prospects and opportunities and are based upon information currently available to the Company and management and is subject to its interpretation of what is believed to be significant factors affecting the businesses, including many assumptions regarding future events. Such forward-looking statements include statements regarding, among other things:

- our ability to produce, sustain and expand sales of our products;
- our ability to develop, acquire and/or introduce new products;
- our projected future sales, profitability and other financial metrics;
- our future financing plans;
- our anticipated needs for working capital;
- the anticipated trends in our industry;
- acquisitions of other companies or assets that we might undertake in the future;
- our operations in the United States and abroad, and the domestic and foreign regulatory, economic and political conditions; and
- competition existing today or that will likely arise in the future.

Forward-looking statements, which involve assumptions and describe our future plans, strategies and expectations, are generally identifiable by use of the words “may,” “should,” “expect,” “anticipate,” “estimate,” “believe,” “intend,” “seek,” or “the negative of these words or other variations on these words or comparable terminology. Actual results, performance, liquidity, financial condition and results of operations, prospects and opportunities could differ materially from those expressed in, or implied by, these forward-looking statements as a result of various risks, uncertainties and other factors, including the ability to raise sufficient capital to continue the Company’s operations. Actual events or results may differ materially from those discussed in forward-looking statements as a result of various factors, including, without limitation, the risks outlined under “Risk Factors” and matters described in this Annual Report generally. In light of these risks and uncertainties, there can be no assurance that the forward-looking statements contained in this Annual Report will in fact occur. Potential investors should not place undue reliance on any forward-looking statements. Except as expressly required by the federal securities laws, there is no undertaking to publicly update or revise any forward-looking statements, whether as a result of new information, future events, changed circumstances or any other reason.

The specific discussions in this Annual Report about the Company include financial projections and future estimates and expectations about the Company’s business. The projections, estimates and expectations are presented in this Annual Report only as a guide about future possibilities and do not represent actual amounts or assured events. All the projections and estimates are based exclusively on the Company management’s own assessment of the business, the industry in which it works and the economy at large and other operational factors, including capital resources and liquidity, financial condition, fulfillment of contracts and opportunities. The actual results may differ significantly from the projections.

Potential investors should not make an investment decision based solely on the Company’s projections, estimates or expectations.

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PART I

In this Annual Report on Form 10-K, unless the context requires otherwise, the terms “we”, “our”, “us”, “Retrophin” and the “Company” refer to Retrophin, Inc., a Delaware corporation, as well as our direct and indirect subsidiaries.

ITEM 1. BUSINESS

Those statements in the following discussion that are not historical in nature should be considered to be forward looking statements that are inherently uncertain. Actual results and the timing of the events may differ materially from those contained in these forward looking statements due to a number of factors, including those discussed in the “Cautionary Statement Regarding Forward-Looking Statements” and “Risk Factors” set forth elsewhere in this Annual Report.

Overview

We are a fully integrated biopharmaceutical company with approximately 130 employees headquartered in San Diego, California, focused on the development, acquisition and commercialization of therapies for the treatment of serious, catastrophic or 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 serious, catastrophic or rare diseases and that we believe offer attractive growth characteristics.

We currently sell the following three products:

Chenodal® (chenodeoxycholic acid) 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) is approved in the United States for the treatment of bile acid synthesis disorders due to single enzyme defects and is further indicated for adjunctive treatment of patients with peroxisomal disorders.

Thiola® (tiopronin) is approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.

Our Strategy

Our goal is to become a leading biopharmaceutical company specializing in the development and commercialization of therapies that deliver significant value for patients with serious, catastrophic or rare diseases. In order to achieve our goal, we intend to:

Expand our product pipeline. We intend to expand our product pipeline by pursuing additional acquisitions of pharmaceutical products that have the potential to have a profound impact on patients’ lives. We believe that there are multiple drugs for treating life-threatening diseases that may be neglected by other pharmaceutical companies. We believe that we can create value by acquiring certain of these products.

Focus on developing products to treat rare diseases characterized by severe unmet medical needs. We focus on potentially transformational orphan drug candidates in order to leverage our development and commercialization capabilities in rare disease. We believe that drug development for orphan drug markets is particularly attractive because relatively small clinical trials can demonstrate the large clinical effects expected with transformational therapies. Furthermore, the regulatory and commercial models for orphan drugs are well established. Finally, we believe that our research, development, and commercialization capabilities are well suited to the orphan drug market and represent distinct competitive advantages.

Develop a sustainable pipeline by employing disciplined decision criteria in the evaluation of potential in-licensing candidates. We seek to build a sustainable product pipeline by employing multiple therapeutic approaches and by developing or acquiring orphan drug candidates. We will seek to augment our internally developed pipeline projects by selectively and strategically acquiring pipeline assets that will add value to the portfolio. We intend to mitigate risk by employing rigorous decision criteria, favoring drug candidates that have undergone at least some clinical study. Our decision to acquire rights to a drug candidate also depends on the scientific merits of the available clinical data; the identifiable orphan patient population; the economic terms of any proposed acquisition of rights; the amount of capital required to develop the asset; and the economic potential of the drug candidate, should it be commercialized. We believe this strategy minimizes our clinical development risk and allows us to accelerate the development and potential commercialization of current and future drug candidates.

Evaluate the commercialization strategies on a product-by-product basis to maximize the value of each. As we move our drug candidates through development toward regulatory approval, we will evaluate several options for each drug candidate's commercialization strategy. These options include building our own internal sales force; entering into joint marketing partnerships with other pharmaceutical or biotechnology companies, whereby we jointly sell and market the product; and out-licensing our products, whereby other pharmaceutical or biotechnology companies sell and market our product and pay us a royalty on sales. Our decision will be made separately for each product and will be based on a number of factors including capital necessary to execute on each option, size of the market and terms of potential offers from other pharmaceutical and biotechnology companies.

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Our Product Candidates and Products on the Market

The following table summarizes the status of our products on the market, product candidates and preclinical programs, each of which are described in further detail below.

Products on the Market:

Chenodal (chenodiol tablets)

Chenodal is a synthetic oral form of chenodeoxycholic acid, a naturally occurring primary bile acid synthesized from cholesterol in the liver, indicated for the treatment of radiolucent stones in well-opacifying gallbladders in 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 Food and Drug Administration (the "FDA") in 1983 for the management of gallstones but its marketing was later discontinued due to lack of commercial success. In 2009 an Abbreviated New Drug Application, or ANDA, for Chenodal submitted by Nexgen Pharma was approved by the FDA for the treatment of gallstones; Chenodal® is private label manufactured for Manchester Pharmaceuticals LLC ("Manchester") under this ANDA. Manchester subsequently obtained Orphan Drug Designation for Chenodal for the treatment of cerebrotendinous xanthomatosis ("CTX") in 2010.

There are currently no FDA approved products for CTX. While not approved, Chenodeoxycholic acid ("CDCA") has been used as the standard of care for CTX for over three decades. We are working to obtain FDA approval of Chenodal for the treatment of CTX, a rare autosomal recessive lipid storage disease. The prevalence of CTX is estimated in the literature to be as high as 1 in 70,000 in the overall population. Pathogenesis of CTX involves deficiency of the enzyme 27-hydroxylase (encoded by the gene CYP27A1), a rate-limiting enzyme in the synthesis of primary bile acids, including 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.

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### Cholbam

The FDA approved Cholbam 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. Approximately 30 patients have transitioned from the open label extension trial to commercial product. The estimated incidence of bile acid synthesis disorders due to single enzyme defects is 1 to 9 per million live births.

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

- sterol 27-hydroxylase (presenting as cerebrotendinous xanthomatosis, CTX) deficiency;

• 2- (or alpha-) methylacyl-CoA racemase (AMACR) deficiency;

• cholesterol 7 alpha-hydroxylase (CYP7A1) deficiency.

### 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 have built a sales force to promote Thiola to targeted physicians.

### Product Candidates:

#### Sparsentan

Sparsentan, also known as RE-021, is an investigational therapeutic agent which acts as both a potent angiotensin receptor blocker (“ARB”), as well as a selective endothelin receptor antagonist (“ERA”), with selectivity toward endothelin receptor type A. We are 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 FDA approved treatments for FSGS and the off-label armamentarium is limited to ACE/ARBs, steroids, and immunosuppressant agents, which are effective in only a subset of patients. We estimate that there are at least 40,000 FSGS patients in the United States. We are currently enrolling patients for the DUET Phase 2 clinical study of sparsentan for the treatment of FSGS and we anticipate having a top line data read out in the third quarter of 2016. Depending on the robustness of the data obtained in the DUET study, we may be able to support an application for accelerated approval for sparsentan on the basis of proteinuria as a surrogate endpoint. Sparsentan was granted orphan drug designation in the U.S. and EU in January and November 2015, respectively.

#### RE-024

We are developing 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. PKAN is estimated to affect 1 to 3 persons per million. There are currently no viable treatment options for patients with PKAN. RE-024 is a phosphopantothenate prodrug therapy that aims to restore levels of this key substrate in PKAN patients. Certain international health regulators have approved the initiation of dosing RE-024 in PKAN patients under physician-initiated studies in accordance with local regulations in their respective countries. The Company filed a U.S. IND for RE-024 with the FDA in the first quarter of 2015 to support the commencement of a Company-sponsored Phase 1 study, which was successfully completed during the year. RE-024 was granted orphan drug designation from the FDA in May 2015 and was granted fast track designation in June 2015. On February 24, 2016, we announced RE-024 was granted orphan drug designation from the European Commission. The Company has begun interacting with the FDA for a potential registration trial.

#### RE-034 (Tetracosactide Zinc)



RE-034 is a synthetic hormone analog of the first 24 amino acids of the 39 amino acids contained in ACTH incorporated into a novel formulation developed by the Company. RE-034 exhibits the same physiological actions as endogenous ACTH by binding to all five melanocortin receptors (pan-MCR), resulting in its anti-inflammatory and immunomodulatory effects. Retrophin has successfully formulated and manufactured RE-034 at proof-of-concept scale using a novel formulation process that allows modulation of the release of the active ingredient from the site of administration. Retrophin continues preclinical development of RE-034 to enable multiple strategic options.

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### Preclinical:

#### NGLY1

The Company entered into a research collaboration with the Grace Wilsey Foundation and the Warren Family Research Center for Drug Discovery and Development at the University of Notre Dame surrounding the development of a novel therapeutic for patients with NGLY1 deficiency, a rare genetic disorder. NGLY1 deficiency is 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 a variety of symptoms, including global developmental delay, movement disorder, seizures, and ocular abnormalities. Under this collaboration, the Grace Wilsey Foundation will provide support and funding to Retrophin to enable discovery efforts that aim to validate and address a new molecular target that may be relevant to NGLY1 deficiency. The Warren Family Research Center for Drug Discovery and Development at the University of Notre Dame will provide funding and in-kind research support to help Retrophin advance this program.

#### Competition

The pharmaceutical and biotechnology industries are intensely competitive and subject to rapid and significant technological change. Most of our competitors are larger than us and have substantially greater financial, marketing and technical resources than we have.

The development and commercialization of new products to treat orphan diseases is highly competitive, and we expect considerable competition from major pharmaceutical, biotechnology and specialty pharmaceutical companies. As a result, there are, and will likely continue to be, extensive research and substantial financial resources invested in the discovery and development of new orphan drug products.

We are a company with a limited history of operations. Many of our competitors have substantially more resources than we do, including both financial and technical. Our competition will be determined in part by the potential indications for which drugs are developed and ultimately approved by regulatory authorities. The speed with which we can develop products, complete pre-clinical testing, clinical trials, approval processes, and supply commercial quantities to market are expected to be important competitive factors. We expect that competition among products approved for sale will be based on various factors, including product efficacy, safety, reliability, availability, price, reimbursement, and patent position.

#### Chenodal

Statins lower cholesterol and have been studied as a treatment for CTX. However, statins deplete CoQ10 and thereby alter mitochondrial function, which is a theoretical concern because abnormal mitochondrial metabolism has been reported in CTX. Although data are sparse, statin monotherapy appears to have little or no benefit for CTX. However, statins may be useful for lowering cholestanol levels when combined with CDCA, and there is limited evidence that they provide additional clinical benefit over CDCA treatment alone.

#### Cholbam

In the U.S. there are currently no competitors.

#### Thiola

D-penicillamine is the only other prescription medication FDA approved for the treatment of cystinuria.

D-penicillamine forms a penicillamine-cysteine disulfide that is 50 times more soluble than cystine. In uncontrolled trials and observational studies, penicillamine decreases stone size or dissolves stones in up to 75 percent of patients. The use of D-penicillamine is often limited by a relatively high incidence of side effects, such as fever, rash, abnormal taste, arthritis, leukopenia, aplastic anemia, hepatotoxicity, and pyridoxine (vitamin B6) deficiency. In addition, patients treated with penicillamine may develop proteinuria (usually due to membranous nephropathy), typically within the first 6 to 12 months of therapy, or, less commonly, crescentic glomerulonephritis. Given the high incidence of side effects, drug therapy may be discontinued once preexisting stones have dissolved. Additional courses can be given if stones recur. If penicillamine is to be used long term, pyridoxine supplementation (50 mg/day) is required. Captopril is not FDA approved for the treatment of cystinuria but has been prescribed for patients with cystinuria. The proportion of orally administered captopril that appears in the urine is low. Thus, the doses of captopril required to reduce cystine excretion (more than 150 mg/day) may not be tolerated because of hypotension. In addition, the efficacy of captopril as a treatment for cystinuria remains unproven. Thus, its use is typically limited to patients who cannot tolerate other cystine-binding agents.

Imprimis Pharmaceuticals, Inc., a specialty pharmaceutical company, announced plans to introduce a compounded form of tiopronin, the active ingredient in Thiola, in combination with potassium citrate. Compounded therapies are not subjected to the same level of safety and efficacy evaluation and may not offer the same therapeutic outcome for patients. There is no clinical data to support the compatibility of fixed dosing of tiopronin with potassium citrate. Fixed-dose combinations of therapies containing potassium are generally avoided due to the potential for fluctuations in serum potassium, which may cause serious adverse outcomes including cardiac events.

#### Sparsentan

There are currently no products approved for FSGS in Europe or the United States. Generally, patients with primary FSGS are treated using glucocorticoids such as prednisone as initial therapy when proteinuria is  $>3.5$  g/day and accompanied by hypoalbuminemia  $<3.5$  g/dL ( $<35$  g/L). Depending upon the response to and the toxicity from this therapy, the duration of prednisone therapy can vary from as short as 8 to 12 weeks to as long as one year. Some patients treated with glucocorticoids have only a transient remission or no remission whatsoever.

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RE-024

There are currently no viable treatment options for patients with PKAN.

RE-034 (Tetracosactide Zinc)

Questcor's H.P. Acthar Gel (repository corticotropin injection) is a highly purified sterile preparation of the adrenocorticotrophic hormone in 16% gelatin. Acthar is the only approved long-lasting ACTH medication in the U.S. H.P. Acthar Gel is indicated for several diseases which would be a competitor for any indications we pursue.

Amphastar's Cortrosyn® (cosyntropin) for injection use is a sterile lyophilized powder in vials containing 0.25 mg of Cortrosyn® and 10 mg of mannitol. Cortrosyn® is indicated for the ACTH Stimulation Test which measures the ability of the adrenal cortex to respond to ACTH by producing cortisol appropriately. Administration is by intravenous or intramuscular injection. Currently, Cortrosyn is only approved as a diagnostic, not as a drug. Further, Cortrosyn is a short acting formulation of ACTH in contrast to Synacthen Depot and Acthar.

Acquisition of Cholic Acid

In January 2015, the Company announced the signing of a definitive agreement under which it acquired the exclusive option to purchase from Asklepiion Pharmaceuticals, LLC all worldwide rights, titles, and ownership of Cholbam (cholic acid) for the treatment of bile acid synthesis defects, if approved by the Food and Drug Administration (the "FDA"). In March 2015, the FDA approved Cholbam capsules and the Company then exercised its option and acquired from Asklepiion all worldwide rights, titles and ownership of Cholbam, including all related contracts, data assets, intellectual property, regulatory assets and a pediatric priority review voucher (the "PRV").

The total purchase price of the assets was \$91.3 million. The Company paid Asklepiion \$33.4 million in cash, transferred 661,279 shares valued at \$15.8 million and agreed to pay contingent consideration consisting of milestones and tier royalties.

Kolbam in Europe

Kolbam, the bottled and branded name of Cholbam in Europe, was approved in the European Union ("EU") in April 2014. In June 2015, the General Court of the EU annulled the marketing authorization ("MA") for Kolbam due to a labeling conflict with the competitor product Orphacol. The EU Commission approved a new MA with revised labeling on November 20, 2015.

Divestiture of Assets:

Sale of Assets to Turing Pharmaceuticals

On October 13, 2014, the Company entered into a binding Summary Separation Proposal with its then-current Chief Executive Officer. Among other things, the Summary Separation Proposal set forth a summary of the terms for the sale of the Company's Vecamyl, Syntocinon and ketamine licenses and assets to Turing Pharmaceuticals, a company controlled by the former Chief Executive Officer.

On January 9, 2015, the Company entered into a purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals the Sold Assets for a purchase price of \$1.0 million, and pursuant to which Turing Pharmaceuticals also assumed all future liabilities related to the Sold Assets.

On February 13, 2015, the Sellers entered into a purchase agreement with Waldun, pursuant to which the Sellers sold Waldun the Vecamyl Product Rights for a purchase price of \$0.7 million. Waldun in turn sold the Vecamyl Product Rights to Turing Pharmaceuticals. In connection therewith, on February 13, 2015, the Company, together with Manchester, entered into an asset purchase agreement with Turing Pharmaceuticals, pursuant to which the Company sold Turing Pharmaceuticals the Inventory for a purchase price of \$0.3 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Vecamyl Product Rights and the Inventory.

On February 13, 2015, the Company entered into an asset purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals its Oxytocin Assets, including related inventory, for a purchase price of \$1.1 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Oxytocin Assets.

The total impact to the Statement of Operations and Comprehensive Income (Loss) related to the divestitures for 2015 was \$0.9 million. See Note 9. to the financial statements for more information.

Sale of Asset to Sanofi

On July 2, 2015, the Company sold and transferred the PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million is due on each of the first and second anniversaries of the closing. The Company recorded the future short term and long term receivables at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale. The gain from the sale of the asset was approximately \$140.0 million, net of \$4.9 million in fees contractually due as part of the Cholbam acquisition.

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### Licenses and Royalties

#### Ligand License

We have a worldwide license from Ligand for the development, manufacture and commercialization of sparsentan, an ARB and ERA which we are initially developing in connection with the treatment of FSGS. Under the license agreement, Ligand granted us a sublicense under certain of its patents and other intellectual property in connection with the development and commercialization of sparsentan. Under the license agreement, Ligand is obligated to transfer to us certain information, records, regulatory filings, materials and inventory controlled by Ligand and relating to or useful for developing sparsentan. We must use commercially reasonable efforts to develop and commercialize sparsentan in specified major market countries and other countries in which we believe it is commercially reasonable to develop and commercialize such products.

As consideration for the license, we are required to make payments upon the achievement of certain milestones, totaling up to \$109.4 million. Should we commercialize sparsentan or any products containing any of these compounds, we will be obligated to pay Ligand an escalating annual royalty between 15% and 17% of net sales of all such products. Through 2015, we made payments to Ligand of \$3.8 million under the terms of the license agreement. Under the terms of the license agreement, Bristol-Myers Squibb will have a right of first negotiation and Ligand will have a right of second negotiation with respect to any license arrangement for a licensed compound.

The license agreement will continue until neither party has any further payment obligations under the agreement and is expected to continue for approximately 10 to 20 years. Ligand may also terminate the license agreement due to (i) our insolvency, (ii) our material uncured breach of the agreement, (iii) our failure to use commercially reasonable efforts to develop and commercialize sparsentan as described above or (iv) certain other conditions. We may terminate the license agreement due to a material uncured breach of the agreement by Ligand.

In September 2015, the license agreement was amended to facilitate sub-licensing in Asia-Pacific. As consideration for the amendment Retrophin paid \$1.0 million.

#### Thiola® License Agreement

In 2014, the Company entered into a license agreement with Mission Pharmacal Company ("Mission"), in which the Company obtained an exclusive, royalty-bearing license to market, sell and commercialize Thiola (Tiopronin) in the United States and Canada, and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola.

The Company paid Mission an up-front license fee of \$3.0 million and will pay guaranteed minimum royalties during each calendar year the greater of \$2.0 million or 20% of the Company's net sales of Thiola through June 30, 2024. As of December 31, 2015, the present value of guaranteed minimum royalties payable is \$10.9 million, using a discount rate of approximately 11% based on the Company's borrowing rate at that point. As of December 31, 2015, the guaranteed minimum royalties' current and long term liability is approximately \$0.8 million and \$10.1 million, respectively, and is recorded as guaranteed minimum royalty in the consolidated balance sheet. The Company has capitalized \$24.1 million related to the Thiola asset which consists of the up-front license fee, professional fees, present value of the guaranteed minimum royalties and any additional payments through 2015 in excess of minimum royalties.

In October 2015, the license agreement was amended to allow for Retrophin secure enough active pharmaceutical ingredient ("API") to ensure an adequate level safety stock to prevent an interruption in the supply of Thiola and to prepare for a potential reformulation development project.

#### Intellectual Property

The proprietary nature of, and protection for, our product candidates and our discovery programs, processes and know-how are important to our business. We have sought patent protection in the United States and certain other jurisdictions for sparsentan, RE-024, RE-034 and certain other inventions to which we have rights, where available and when appropriate. Our policy is to pursue, maintain and defend patent rights, whether developed internally or licensed from third parties, and to protect the technology, inventions and improvements that are commercially important to the development of our business. We also rely on trade secrets relating to our proprietary technology that may be important to the development of our business.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection for our current and future product candidates and the methods used to develop and manufacture them, as well as successfully defending these patents against third-party challenges. Our ability to stop third parties from making, using, selling, offering to sell, or importing our products depends on the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. We cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our product candidates, discovery programs and processes.

#### Sparsentan (RE-021)

Our patent portfolio for sparsentan is comprised of two distinct patent families, both of which are exclusively licensed from Ligand. One of these patent families is owned by Bristol-Myers Squibb Company (“BMS”), which exclusively licensed it to Ligand (the “BMS patent family”), and the other is owned by Ligand (the “Ligand patent family”).

The BMS patent family is directed to sparsentan and structural analogs thereof, and to pharmaceutical compositions containing sparsentan or a structural analog thereof. As of December 31, 2016, this patent family included three U.S. patents (U.S. Patent Nos. 6,638,937, which we refer to herein as the ‘937 patent; 6,835,741; and 6,852,745), of which one (U.S. Patent No. 6,638,937) claims sparsentan and pharmaceutical compositions that contain

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sparsentan. In addition, as of December 31, 2016, this patent family included a granted European patent and a granted Chinese patent. With the exception of the '937 patent, which the U.S. Patent and Trade Office ("USPTO") has determined is entitled to 175 days of patent term adjustment, we expect all U.S. and foreign patents in this patent family to expire in July 2019. In view of the USPTO determination that the '937 patent is entitled to 175 days of patent term adjustment, we expect the '937 patent to expire in December 2019.

The Ligand patent family is directed to methods of using sparsentan in the treatment of glomerulosclerosis. As of December 31, 2016, this patent family included applications pending in the United States (Application Serial No. 14/631,768, filed February 25, 2015), China, Europe, Hong Kong and Japan. We expect any U.S. and foreign patents granted in this patent family to expire in March 2030.

It is possible, assuming that sparsentan achieves regulatory approval and depending upon the date of any such approval, that the term of the '937 patent may be extended up to a maximum of five additional years under the provisions of the Drug Price Competition and Patent Term Restoration Act of 1984, also referred to as the Hatch-Waxman Act. Patent term extension also may be available in certain foreign jurisdictions upon regulatory approval.

### PKAN (RE-024)

Our patent portfolio covering compounds for the treatment of PKAN is comprised of three Retrophin-owned patent families. The first of these three patent families includes patents and patent applications directed to RE-024 and structural analogs thereof, pharmaceutical compositions containing RE-024 or analogs thereof, and methods of using RE-024 or analogs thereof in the treatment of PKAN. As of December 31, 2015, this patent family included two U.S. patents (U.S. Patent No. 8,673,883, issued March 18, 2014, which we refer to herein as the '883 patent, and U.S. Patent No. 9,181,286, issued November 10, 2015), one pending U.S. patent application (Application Serial No. 14/871,450, filed September 30, 2015) and corresponding foreign patent applications pending in Australia, Brazil, Canada, China, Europe, Hong Kong, India, Japan, Korea, Mexico, and Russia. We expect all U.S. and foreign patents in this patent family to expire in April 2033.

Our second PKAN patent family is directed to a chemical genus that encompasses structural analogs of RE-024, but not RE-024 itself. As of December 31, 2015, this patent family was comprised of International Patent Application PCT/US2014/062451, filed October 27, 2014. We expect any U.S. or foreign patent family granted in this patent family to expire in October 2034.

Our third PKAN patent family is directed to a chemical genus that encompasses structural analogs of RE-024, but not RE-024 itself. As of December 31, 2015, this patent family was comprised of a U.S. provisional patent application filed in 2015.

It is possible, assuming that RE-024 achieves regulatory approval and depending upon the date of any such approval, that the term of the '883' patent may be extended up to a maximum of five additional years under the provisions of the Hatch-Waxman Act. Patent term extension also may be available in certain foreign jurisdictions upon regulatory approval. Should we commercialize RE-024, we may be obligated to pay royalties of up to 5% of net sales of all such products.

### RE-034 (Tetracosactide Zinc)

Our patent portfolio for RE-034 is comprised of a U.S. provisional patent application filed in February 2015.

### Regulatory Exclusivity

If we obtain marketing approval for sparsentan, RE-024, RE-034, or other drug candidates in the United States or in certain jurisdictions outside of the United States, we may be eligible for regulatory protection. For example, in the U.S. an FDA approved product may be eligible to receive five years of new chemical entity exclusivity or, for drugs granted an orphan designation by the FDA, seven years of orphan drug exclusivity. In Europe a new drug product approved by the EMA may receive eight years of data exclusivity and up to 11 years of marketing exclusivity or, in the case of orphan drugs, ten years of data exclusivity. There can be no assurance that we will qualify for any such regulatory exclusivity, or that any such exclusivity will prevent competitors from seeking approval solely on the basis of their own studies. See "Government Regulation" below.

Chenodal



Chenodal received orphan drug designation in the U.S. for the treatment of CTX in 2010. Consequently, if Chenodal gains FDA approval for the treatment of CTX it will have 7 years of marketing exclusivity in the U.S. for that indication. We are not currently pursuing EMA authorization to market Chenodal in Europe.

Cholbam (Kolbam)

Cholbam received orphan drug designation in the U.S. for the treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects and for patients with peroxisomal disorders, and therefore will have marketing exclusivity in the U.S. for these indications until March 2022.

Kolbam, the bottled and branded name of Cholbam in Europe, received marketing authorization in November 2015 from the EMA for the treatment of inborn errors of primary bile acid synthesis, encompassing select single enzyme defects. Kolbam will have marketing exclusivity in Europe for these indications until September 2024.

Thiola

Thiola does not have regulatory exclusivity in the U.S.

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### Trademarks

Our trademark portfolio is comprised a registered U.S. trademark and U.S. and foreign trademark applications for the mark “RETROPHIN”, one U.S. trademark application directed to the Retrophin logo, one registered U.S. trademark and one registered Canadian trademark for the mark “CHENODAL”, one registered U.S. trademark directed to the Chenodal logo, one registered U.S. trademark for the mark “MANCHESTER PHARMACEUTICALS”, one U.S. trademark application for the mark “KEEP IT BELOW THE LINE”, a registered U.S. trademark and foreign trademark applications for the mark “CHOLBAM”, a registered European Community trademark for the mark “KOLBAM”, a registered U.S. trademark for the mark “TOTAL CARE HUB”, a U.S. trademark application directed to the Total Care Hub logo, and a U.S. trademark application directed a leaves logo. In addition, under our license agreement with Mission we have an exclusive license to use Mission’s three registered U.S. trademarks and one registered Canadian trademark for the mark “THIOLA”.

### Trade Secrets

In addition to patents, we rely on trade secrets and know-how to develop and maintain our competitive position. We seek to protect our proprietary data and processes, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors, and partners. These agreements are designed to protect our proprietary information. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems. Trade secrets and know-how can be difficult to protect. Consequently, we anticipate that trade secrets and know-how will, over time, be disseminated within the industry through independent development, the publication of journal articles, and the movement of personnel skilled in the art from academic to industry scientific positions.

### Manufacturing and Distribution

Nexgen Pharma manufactures Chenodal, New Zealand Pharma manufactures the API and Patheon formulates and packages Cholbam, and Mission manufactures Thiola. Dohmen Life Sciences Services (“Dohmen”) is our distributor. We intend to continue to use our financial resources to accelerate development of our drug candidates rather than diverting resources to establish our own manufacturing facilities. We intend to meet our pre-clinical and clinical trial manufacturing requirements by establishing relationships with third-party manufacturers and other service providers to perform these services for us.

Should any of our drug candidates obtain marketing approval, we anticipate establishing relationships with third-party manufacturers and other service providers in connection with the commercial production of our products. We have some flexibility in securing other manufacturers to produce our drug candidates; however, our alternatives may be limited due to proprietary technologies or methods used in the manufacture of some of our drug candidates.

### Sales and Marketing

During fiscal 2015, we continued to build a specialty sales force to market our products. In order to commercialize our clinical drug candidates if and when they are approved for sale in the United States or elsewhere, we will need to increase our marketing, sales and distribution capabilities.

### Commercialization

Through deep understanding of patient and healthcare provider needs, we believe the Company is able to:

- serve patients living with rare disease that have limited treatment options;
- drive optimum performance of its marketed products;
- educate and train healthcare providers about our products and the diseases for which they are approved to treat;
- support access to and reimbursement coverage for our products in the U.S. without significant restrictions; and

minimize the number of patients who discontinue treatment or have low compliance with our products by providing patients with support services and disease education, to the extent and in the manner permitted under applicable laws, to help them maximize the benefits of treatment.

Our U.S. commercial initiatives are designed to support patients living with rare diseases and clinicians treating these patients. We believe that it is possible to commercialize our products in the U.S. with a relatively small specialty sales force. The primary call points for Thiola include urologists and nephrologists. The primary call points for Cholbam

are gastroenterologists, hepatologists, and metabolic specialists. We do not promote Chenodal with our sales force. Our sales force is differentiated by its high level of experience, averaging more than 15 years in pharmaceutical sales including over five years of experience in rare disease. Our commercial management and operations team has an average of more than 15 years of pharmaceutical experience focused on specialty and rare disease. Our small marketing team, supported by third-party agencies with rare disease experience, drives our commercialization and disease awareness efforts in the U.S. and countries where our products may be approved or available through named patient sales. Specifically, we implement a variety of marketing programs to educate physicians, including direct-to-physician contact by sales representatives, peer-to-peer educational programs, and participation in targeted medical convention programs.

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We distribute our products through one direct to patient pharmacy, Dohmen Life Sciences Services, who also provide our comprehensive patient support services (i.e, the Total Care Hub). This patient support program (for all U.S. commercial products) includes a case-managed approach to patient education, insurance verification and reimbursement support, co-pay and other financial assistance for eligible patients, monitoring and support of adherence, and 24/7 access to pharmacist counseling.

Outside the U.S., including in the EU, we have hired region managers and other field-based employees in certain key countries and plan to hire medical or commercial field-based personnel where needed to engage in educational activities. In certain countries outside the U.S., we have engaged, or plan to engage, local distributors to conduct permitted commercial activities. Our near-term efforts are focused on securing pricing and reimbursement approval for Kolbam/Cholbam.

### Medical Affairs

We have a medical affairs team in the U.S. which supports independent medical education programs and investigator-initiated studies by providing education and financial grants in a number of medical and disease-related areas. The responsibilities of medical affairs personnel also include providing education through the dissemination of medical information and publications, providing support in connection with our post-approval clinical commitments, and assisting in organizing scientific and medical advisory boards to obtain input from experts and practitioners on medical topics relevant to our products and diseases.

### Government Regulation

Regulation by government authorities in the United States and foreign countries is a significant factor in the development, manufacture and marketing of our proposed products and in our ongoing research and product development activities. All of our products will require regulatory approval by government agencies prior to commercialization. In particular, human therapeutic products are subject to rigorous preclinical studies and clinical trials and other approval procedures of the FDA and similar regulatory authorities in foreign countries. Various federal and state statutes and regulations also govern or influence testing, manufacturing, safety, labeling, storage and record-keeping related to such products and their marketing. The process of obtaining these approvals and the subsequent compliance with appropriate federal and state statutes and regulations require the expenditure of substantial time and financial resources.

### FDA Drug Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending new drug applications, or NDAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties and criminal prosecution.

We cannot market a drug product candidate in the United States until the drug has received FDA approval. The steps required before a drug may be marketed in the United States generally include the following:

- completion of extensive pre-clinical laboratory tests, animal studies, and formulation studies in accordance with the FDA's GLP regulations;
- submission to the FDA of an IND for human clinical testing, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials in accordance with GCP requirements to establish the safety and efficacy of the drug for each proposed indication;
- submission to the FDA of an NDA after completion of all pivotal clinical trials;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the active pharmaceutical ingredient, or API, and finished drug product are produced and tested to assess compliance with cGMPs; and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol. Long term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. If the FDA raises concerns or questions about the conduct of the trial, such as whether human research subjects will be exposed to an unreasonable health risk, the IND sponsor and the FDA must resolve any outstanding FDA concerns or questions before clinical trials can proceed.

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Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with federal regulations, including GCP requirements, as well as under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, for approval at each site at which the clinical trial will be conducted. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a limited patient population to determine metabolism, pharmacokinetics, the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 clinical trials, also called pivotal trials, are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases the FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 clinical trial with other confirmatory evidence may be sufficient in rare instances where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the United States. The NDA must include the results of all preclinical, clinical and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture and controls. The cost of preparing and submitting an NDA is substantial. The submission of most NDAs is additionally subject to a substantial application user fee, and the manufacturer and/or sponsor under an approved NDA are also subject to annual product and establishment user fees. These fees are typically increased annually. The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs. Most such applications for standard review drug products are reviewed within 10 to 12 months; most applications for priority review drugs are reviewed in six to eight months. Priority review can be applied to drugs to treat serious conditions that the FDA determines offer significant improvement in safety or effectiveness. The review process for both standard and priority review may be extended by the FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission.

The FDA may also refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an advisory committee—typically a panel that includes clinicians and other experts—for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with cGMPs is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing, or information, in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require risk evaluation and mitigation strategies ("REMS") to ensure that the benefits of the drug outweigh the potential risks. REMS can include a medication guide, a communication plan for healthcare professionals and elements to assure safe use, such as special training and certification requirements for individuals who prescribe or dispense the drug, requirements that patients enroll in a registry and other measures that the FDA deems necessary to assure the safe use of the drug. The requirement for REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs. Such supplements are typically reviewed within 10 months of receipt.

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### Orphan Drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting an NDA. After the FDA confers orphan drug status, the generic identity of the drug and its potential orphan indication are disclosed publicly by the FDA. Orphan drug designation in and of itself does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular indication with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the U.S. for that product, for that indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Prior to FDA approval, orphan designation provides incentives for sponsors including tax credits for clinical research expenses, the opportunity to obtain government grant funding to support clinical research, and an exemption from FDA user fees.

### Fast Track Designation

Fast track is a process designed by the FDA to facilitate the development of drugs to treat serious conditions through expediting their review. The purpose is to get important new drugs to patients earlier. Fast Track addresses a broad range of serious conditions. Determining whether a condition is serious is a matter of judgment, but generally is based on whether the drug will have an impact on such factors as survival, day-to-day functioning, or the likelihood that the condition, if left untreated, will progress from a less severe condition to a more serious one.

A drug that receives Fast Track designation is eligible for some or all of the following:

- More frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval;

- More frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers;

- Eligibility for Accelerated Approval and Priority Review, if relevant criteria are met; and

- Rolling Review, which means that a drug company can submit completed sections of its Biologic License Application (BLA) or NDA for review by FDA, rather than waiting until every section is completed before the entire application can be reviewed. BLA or NDA review usually does not begin until the drug company has submitted the entire application to the FDA.

Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

### Accelerated Approval

Under the FDA's accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis.

All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by FDA.

The Hatch-Waxman Amendments: Orange Book Listing



In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

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A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

### Post-Approval Requirements

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet and social media. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, surveillance to monitor the effects of an approved product, or restrictions on the distribution or use of the product. In addition, quality-control, drug manufacture, packaging and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- or
- injunctions or the imposition of civil or criminal penalties.

### Pricing and Reimbursement

A portion of our end-user demand for our drugs comes from patients covered under Medicaid, Medicare and other federal and state government-related programs such as TRICARE and the Department of Veterans Affairs, or the VA. As required by Federal regulations, we will provide rebates and discounts in connection with these programs. Our commercial success depends in significant part on the extent to which coverage and adequate reimbursement for these products will be available from third-party payers, including government health administration authorities, private health insurers and other organizations. Third-party payers determine which medications they will cover and establish reimbursement levels. Even if a third-party payer covers a particular product, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payers to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement is critical to product acceptance.

Government authorities and other third-party payers are developing increasingly sophisticated methods of controlling healthcare costs, such as by limiting coverage and the amount of reimbursement for particular medications.

Increasingly, third-party payers are requiring that drug companies provide them with predetermined discounts from list prices as a condition of coverage, are using restrictive formularies and preferred drug lists to leverage greater discounts in competitive classes, and are challenging the prices charged for medical products. Third party payers also are carefully evaluating the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy, which may require us to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products. Further, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payers in the United States. Therefore, coverage and reimbursement for drug products can differ significantly from payer to payer. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payer separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

In addition, it is possible that future legislation in the United States and other jurisdictions could be enacted which could potentially impact the coverage and reimbursement rates for our products and also could further impact the levels of discounts and rebates paid to federal and state government entities. Any legislation that impacts these areas could impact, in a significant way, our ability to generate revenues from sales of products that, if successfully developed, we bring to market.

There have been a number of enacted or proposed legislative and regulatory changes affecting the healthcare system and pharmaceutical industry that could affect our commercial success. For example, in March 2010, President Obama signed into law the Patient Protection and Affordable Care

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Act, as amended by the Health Care and Education Reconciliation Act of 2010, (collectively, the “Health Care Reform Law”) a law intended to, among other things, broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on pharmaceutical and medical device manufacturers and impose additional health policy reforms. Among other things, the Health Care Reform Law expanded manufacturers’ rebate liability under the Medicaid Drug Rebate Program, which could increase the amount of Medicaid drug rebates manufacturers are required to pay to states. The Health Care Reform Law also expanded the types of entities eligible to receive discounted 340B pricing, although, with the exception of children’s hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs used in orphan indications. The Health Care Reform Law also imposed a significant annual fee on companies that manufacture or import certain branded prescription drug products. Furthermore, the Health Care Reform Law changed the Medicare Part D coverage gap discount program by requiring manufacturers to provide a 50% point-of-sale-discount off the negotiated price of applicable brand drugs to certain eligible beneficiaries during their coverage gap period as a condition for the manufacturers’ outpatient drugs to be covered under Medicare Part D.

In addition, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. For example, in August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least \$1.2 trillion for fiscal years 2012 through 2021, triggering the legislation’s automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and will stay in effect through 2024 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals and imaging centers.

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.

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 bills designed to, among other things, increase drug pricing transparency, review relationships between pricing and manufacturer patient assistance programs, and reform government program drug reimbursement methodologies. We expect that the Health Care Reform Law, as well as other federal and state healthcare reform measures that have been and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any of our products, and could seriously harm our future revenues.

In addition, it is possible that future legislation in the United States and other jurisdictions could be enacted which could potentially impact the reimbursement rates for the products we are developing and may develop in the future and also could further impact the levels of discounts and rebates paid to federal and state government entities. Any legislation that impacts these areas could impact, in a significant way, our ability to generate revenues from sales of products that, if successfully developed, we bring to market.

### Health Care Regulatory Laws

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain business practices in the pharmaceutical industry in recent years. These laws include, without limitation, anti-kickback statutes and false claims laws, data privacy and security laws, and transparency laws regarding payments or other items of value provided to healthcare providers.

The federal healthcare 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 healthcare item or service reimbursable under Medicare, Medicaid, or other

federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers, among others, on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exceptions and safe harbors are drawn narrowly, and practices that involve remuneration that may induce prescribing, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the anti-kickback statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the federal anti-kickback statute has been violated. Additionally, Health Care Reform Law amended the federal anti-kickback statute to a stricter standard 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. In addition, the Health Care Reform Law codified case law 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 federal civil False Claims Act. Federal false claims laws, including the civil 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 have a false claim paid. This includes claims made to programs where the federal government reimburses, such as Medicaid, as well as programs where the federal government is a direct purchaser, such as when it purchases off the Federal Supply Schedule. The False Claims Act contains qui tam provisions, which allow a private individual, or relator, to bring a civil action on behalf of the federal government alleging that the defendant submitted a false claim to the federal government, and to share in any monetary recovery. In recent years, the number of suits brought by private individuals has increased dramatically. For example, pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to

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pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate federal false claims laws.

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 you 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, or 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 Health Care Reform Law 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, or 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.

Additionally, the federal Physician Payments Sunshine Act within the Health Care Reform Law, and its implementing regulations, require that certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to annually report 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. Certain states also require implementation of commercial compliance programs and compliance with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; impose restrictions on marketing practices; require the registration of sales representatives; or require drug manufacturers to track and report information related to payments, gifts and other items of value to physicians and other healthcare providers. We complied with the Sunshine Act in the first quarter of 2015, as was required.

In addition, several states now require prescription drug companies to report expenses relating to the marketing and promotion of drug products and to report gifts and payments to health care providers and entities in these states. Other states prohibit various other marketing-related activities. Still other states require the posting of information relating to clinical studies and their outcomes. Certain states, such as California and Connecticut, require manufacturers to implement compliance programs and/or marketing codes. Other states, such as Massachusetts and Vermont, impose restrictions on manufacturer marketing practices and require tracking and reporting of gifts, compensation, and other remuneration to healthcare professionals and entities. Several additional states are considering similar proposals. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties.

If our operations are found to be in violation of any of the health regulatory laws described above or any other laws that apply to us, we may be subject to significant penalties, including imprisonment, criminal fines, civil monetary penalties, administrative penalties, disgorgement, and exclusion from participation in federal healthcare programs, contractual damages, injunctions, recall or seizure of products, total or partial suspension of production, reputational harm, administrative burdens, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

#### Foreign Regulation

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain approval by the comparable regulatory authorities of foreign countries before we can commence clinical trials and approval of foreign countries or economic areas, such as the European Union, before we may market products in those countries or areas. The approval process and requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from place to place, and the time may be longer or shorter than that required for FDA approval.

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### Other Laws and Regulatory Processes

We are subject to a variety of financial disclosure and securities trading regulations as a public company in the United States, including laws relating to the oversight activities of the Securities and Exchange Commission (“SEC”), and NASDAQ rules under which the Company’s stock is listed. In addition, the Financial Accounting Standards Board (“FASB”), the SEC, and other bodies that have jurisdiction over the form and content of our accounts, our financial statements and other public disclosure are constantly discussing and interpreting proposals and existing pronouncements designed to ensure that companies best display relevant and transparent information relating to their respective businesses.

Our present and future business has been and will continue to be subject to various other laws and regulations. Various laws, regulations and recommendations relating to safe working conditions, laboratory practices, the experimental use of animals, and the purchase, storage, movement, import and export and use and disposal of hazardous or potentially hazardous substances used in connection with our research work are or may be applicable to our activities. Certain agreements entered into by us involving exclusive license rights or acquisitions may be subject to national antitrust regulatory control, the effect of which cannot be predicted. The extent of government regulation which might result from future legislation or administrative action, cannot accurately be predicted.

### Available Information

Our website address is [www.retrophin.com](http://www.retrophin.com). We post links on our website to the following filings as soon as reasonably practicable after they are electronically filed with or furnished to the SEC: annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, proxy statements, and any amendments to those reports filed or furnished pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended. All such filings are available through our website free of charge. Our filings may also be read and copied at the SEC’s Public Reference Room at 100 F Street, NE, Washington, DC 20549. Information on the operation of the Public Reference Room may be obtained by calling the SEC at 1-800-SEC-0330. The SEC also maintains an internet site at [www.sec.gov](http://www.sec.gov) that contains reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC.

### Item 1A. Risk Factors

Our business, as well as an investment in our common stock, is highly speculative in nature and involves a high degree of risk. Our securities should be purchased only by persons who can afford to lose their entire investment. Carefully consider the risks and uncertainties described below together with all of the other information included herein, including the financial statements and related notes, before deciding to invest in our common stock. If any of the following risks actually occur, they could adversely affect our business, prospects, financial condition and results of operations. In such event(s), the market price of our common stock could decline and result in a loss of part or all of your investment. Accordingly, prospective investors should carefully consider, along with other matters referred to herein, the following risk factors in evaluating our business before purchasing any shares of our common stock.

#### 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;

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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.

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 by the state program for use of any drug for which supplemental rebates are not being paid. Managed care organizations continue to seek price discounts and, in some cases, to impose restrictions on the coverage of particular drugs. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding constraint on prices and reimbursement for our products.

We may not be able to rely on orphan drug exclusivity for Cholbam/Kolbam or any of our product candidates. 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 U.S. and EU. 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 products 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 what had previously been granted 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 affected products may decline materially.

Under the Hatch-Waxman Amendments of the FDC act, a pharmaceutical manufacturer may file an abbreviated new drug application ("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 generic entrants, as the ANDA and NDA for these drug products have no remaining patent or nonpatent exclusivity. If a generic version is approved, sales of our product would be negatively impacted, which could 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 Thiola, and Cholbam. 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

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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, Thiola or Cholbam 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, Thiola and Cholbam 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, Thiola and/or Cholbam 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, if any, could be adversely affected and our business may suffer.

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

### 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.

Our efforts to develop certain of our product candidates are at an early stage. 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. We cannot assure that any future clinical trials of sparsentan, RE-024 and/or RE-034 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. A failure of one or more of our preclinical studies can occur at any stage of testing. The Company filed a U.S. IND and completed a Phase I study for RE-024 in 2015.

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.

There can be no assurance that the DUET Phase 2 clinical study for sparsentan will demonstrate that sparsentan is safe and effective for treating FSGS or that the data will support an application for accelerated approval by the FDA or that the FDA will accept a proteinuria endpoint.

Clinical trials can be lengthy, complex and extremely expensive processes with uncertain results. Our product candidates are intended to treat FSGS and PKAN, each of which is a rare disease. Given that these development candidates are in the early stages of 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. For example, our ability to complete the sparsentan DUET study is dependent upon our ability to enroll FSGS patients. 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. To date, we are not aware of any pharmaceutical product to treat PKAN or FSGS that has been approved by the FDA or EMA specifically for the treatment of these indications. As a result, we cannot be sure what endpoints the FDA and/or EMA will require us to measure in later-stage clinical trials of our product candidates.

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

our preclinical 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;

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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 postmarketing requirements and commitments associated with Cholbam. FDA approval once obtained, may be withdrawn. If the regulatory approval for Thiola, Chenodal and/or Cholbam are withdrawn for any reason, it would have a material adverse impact on our sales and profitability. Further, we face risks relating to the postmarketing 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 acquisition and development of our most advanced product candidates, sparsentan, RE-024 and RE-034. 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 sparsentan, RE-024 and subsequent product candidates for completion of our clinical trials on a timely basis;
- successful completion of pre-clinical and clinical studies;
- 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;
- 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.

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

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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.

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 sparsentan and RE-024, and expect to seek orphan drug designations from the FDA for RE-034, there can be no assurance that there will be any benefits associated with such designation, or that the FDA will grant orphan status. We have filed for orphan designation from the EMA for RE-024 in the EU, and expect to seek orphan designation from the EMA for RE-034. There can be no assurance that we will successfully obtain such designations. If we are unable to secure orphan status in either Europe or the United States it may have a material negative effect on our share price.

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. Obtaining orphan drug exclusivity for RE-034, RE-024, and sparsentan may be important to the product candidate's success. Even if we obtain 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 Research Programs

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

The success of our products is dependent upon achieving and maintaining market acceptance. Commercializing products is time consuming, expensive and unpredictable. There can be no assurance that we will be able to, either by ourselves or in collaboration with our partners or through our licensees, successfully commercialize new products or 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 sparsentan, RE-024 and RE-034 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;

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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 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 candidates 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 technologies marketed by our competitors.

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. RE-024 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 RE-024 for the treatment of PKAN. Sparsentan is covered by U.S. Patent No. 6,638,937, which expires in 2019 and to which we have an exclusive license. Our RE-034 formulation is covered by a U.S. provisional patent application we filed in February 2015.

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 Hatch-Waxman Amendments to the Federal Food, Drug, and Cosmetic Act (the "FDC Act") or seven years regulatory exclusivity via the orphan drug provisions of the FDC Act. In addition, we may be able to obtain up to five years patent term extension (to compensate for regulatory approval delay) for a patent covering such a product.

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

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

We have negotiated a license agreement with Ligand Pharmaceuticals for the rights to sparsentan which we are initially developing for the treatment of FSGS. Further, 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 cannot be certain when or if we will file for patent protection for different indications for sparsentan, if we would be successful in obtaining these patents, or if we would be able to enforce these patents. If we are unsuccessful in obtaining additional patents covering the use of sparsentan for treating FSGS, we may not be able to stop competitors

from marketing sparsentan following the latter of expiration of our sparsentan composition of matter patent (i.e. U.S. Patent No. 6,638,937) and expiration of the regulatory exclusivity afforded to sparsentan upon NDA approval. 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

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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.

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

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 sparsentan, RE-024 and RE-034, or any other product candidate that we develop, restrict or regulate post-approval activities and affect our ability to profitably sell sparsentan, RE-024 and RE-034 or any other product candidate for which we obtain marketing approval.

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.

For example, in March 2010, President Obama signed Health Care Reform Law, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Health Care Reform Law 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. It is likely the Health Care Reform Law will continue to put pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase regulatory burdens and operating costs.

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:

- 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. 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 bills designed to, among other things, increase drug pricing transparency, review relationships between pricing and manufacturer patient assistance programs, and reform government program drug reimbursement methodologies. 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.

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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 FSGS and PKAN, 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 FSGS and PKAN 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 FSGS and PKAN in the study populations accurately reflect the prevalence of these diseases in the broader world population. If our estimates of the prevalence of FSGS or PKAN or of the number of patients who may benefit from treatment with sparsentan and RE-024 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 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 sparsentan RE-024 and RE-034 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 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

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authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect regulatory approval and supplies of our product candidates.

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

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

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

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

**Risks Related to Our Business**

Our limited operating history makes it difficult to evaluate our current business and 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 in the number of our employees and the scope of our operations. We began 2014 with 26 employees and ended the current calendar year with approximately 130 employees, having 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 persuade adequate numbers of physicians to prescribe our products;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;
- unforeseen costs associated with expanding our own sales and marketing team for new products or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize competitive products.

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

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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:

- complete enrollment in the Phase 2 DUET trial of sparsentan for the treatment of FSGS;

- continue our ongoing clinical development of RE-024 for the treatment of PKAN;

- continue our ongoing clinical development of RE-034;

- continue the research and development of additional product candidates;

- expand our sales and marketing infrastructure to commercialize Cholbam 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 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, research and development expenses to increase in connection with our ongoing activities, particularly as we complete Phase 2 clinical studies of sparsentan, and Phase I clinical studies of RE-024, and as we continue toward possible Phase 1 clinical studies of RE-034 and for any later-stage clinical trials of our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers, and product distribution. We currently have no additional commitments or arrangements for any additional financing to fund the research and development and commercial launch of our product candidates. Management believes the Company's ability to continue its operations depends on its ability to sustain and grow revenue, results of operations and its ability to access capital markets when necessary to accomplish its strategic objectives. Management believes that we may incur losses in the immediate future. For the year ended December 31, 2015, the Company 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. The Company expects to finance its 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 sparsentan, RE-024 and RE-034 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;

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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; 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;
- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this "Risk Factors" section.

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

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

We intend to expand our product pipeline by pursuing acquisition of pharmaceutical products. If an acquisition is consummated, 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.

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

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

We have liability insurance policies for our clinical trials in the geographies in which we are conducting trials. The amount of insurance that we currently hold may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost and we may not be able to obtain insurance coverage that will be adequate to satisfy any liability that may arise. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or a series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our available cash and adversely affect our business.

We are involved in various 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 various litigation matters, each described above in Part II, Item 1 “Legal Proceedings”. Although we intend to vigorously defend any claims for which we have been named as a defendant, 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 claims. Any such payments or settlement arrangements could have material adverse effects on our business, operating results or financial condition. Even if the pending claims are not successful, litigation with respect to such claims 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.

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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, advertising, promotion 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 regional, national, 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 Federal Food, Drug, and Cosmetic 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.

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 Health Care Reform Law, 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 Health Care Reform Law 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.

Many states also have statutes or regulations similar to the federal anti-kickback law and false claims and civil monetary penalties laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, which apply regardless of the payer. 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. 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 Health Care Reform Law 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

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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 you 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.

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. The Federal Health Insurance Portability and Accountability Act of 1996 ("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.

Additionally, the federal Physician Payments Sunshine Act within the Health Care Reform Law, 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.

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, imprisonment, injunctions, recall or seizure of products, 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:

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our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;

the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable regulatory authorities for approval;

our inability to demonstrate that a product candidate's benefits outweigh its risks;

our inability to demonstrate that the product candidate presents an advantage over existing therapies;

the FDA's or comparable regulatory authorities' disagreement with the manner in which we interpret the data from preclinical studies or clinical trials;

failure of the third-party manufacturers with which we contract for clinical or commercial supplies to satisfactorily complete an FDA pre-approval inspection of the facility or facilities at which the product is manufactured to assess compliance with the FDA's cGMP regulations to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and

a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. The FDA and non-United States regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

### Risks Related to our Indebtedness and Investments

Our indebtedness could adversely affect our financial condition.

As of December 31, 2015, 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.

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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 other indebtedness of the Company in excess of \$10 million (other than indebtedness that is non-recourse to the Company); or
- certain types of bankruptcy or insolvency involving the Company.

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.

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 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

We lease the following locations to conduct our business:

Location	Address	Lease Expiration	Square Feet
San Diego, California (Principal Executive Office)	12255 El Camino Real, Suite 250	December 31, 2017	11,397
Cambridge, Massachusetts	301 Binney Street	December 31, 2016	13,985
New York, New York	777 Third Avenue, 22 Floor Suites B & C	November 30, 2018	7,661

We believe these facilities are adequate to conduct our business.

Item 3. Legal Proceedings

On January 7, 2014, the Company sued Questcor Pharmaceuticals, Inc. (“Questcor”) in federal court in the Central District of California (Retrophin, Inc. v. Questcor Pharmaceuticals, Inc., Case No. SACV14-00026-JLS). The Company alleged that Questcor violated antitrust laws in connection with its acquisition of rights to the drug Synacthen, and sought injunctive relief and damages. The Company asserted claims under sections 1 and 2 of the Sherman Act, section 7 of the Clayton Act, California antitrust laws, and California’s unfair competition law. On June 4, 2015, pursuant to the terms of a Confidential Settlement Agreement and Release (the “Settlement Agreement”)

the Company and Questcor filed a Stipulation of Dismissal, dismissing the Company's lawsuit against Questcor. Under the terms of the Settlement Agreement, Questcor paid the Company \$15.5 million, recorded as "Litigation Settlement Gain" in the quarter ended June 30, 2015, and the Company and Questcor granted a mutual release of all claims against the other.

On June 13, 2014, Charles Schwab & Co., Inc. ("Schwab") sued the Company, Standard Registrar and Transfer Company ("Standard"), Jackson Su ("Su"), and Chun Yi Huang ("Huang") in federal court in the Southern District of New York (Charles Schwab & Co. v. Retrophin, Inc., Case No. 14-cv-4294). Su and Huang also asserted cross-claims against the Company and Standard for alleged negligent misrepresentation premised upon an alleged failure to inform them of restrictions on the sale of their Company stock, and impleaded Katten Muchin Rosenman LLP as a third-party defendant. Schwab's claims have been dismissed with prejudice. On September 30, 2015, the Court dismissed Su and Huang's cross-claims and third party claims. The dismissal was with prejudice with respect to Su, but without prejudice with respect to Huang. Huang did not seek leave to replead his claims within the time set by the Court. Accordingly, on November 10, 2015, the Court ordered the case to be closed.

On September 19, 2014, purported shareholders of the Company sued Martin Shkreli, the Company's former Chief Executive Officer, in federal court in the Southern District of New York (Donoghue v. Retrophin, Inc., Case No. 14-cv-7640). The Company is a nominal defendant in this action. The plaintiffs sought, on behalf of the Company, disgorgement of short-swing profits from Mr. Shkreli under section 16(b) of the Securities Exchange Act of 1934 (15 U.S.C. 78(p)(b)). The Court has approved a settlement between the parties, under which Mr. Shkreli is obligated to pay \$2,025,000 to the Company and an additional \$625,000 to Plaintiffs to compensate them for their legal fees. Shkreli has defaulted on the judgment and the Company and the Plaintiffs are taking steps to collect it.

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On October 20, 2014, a purported shareholder of the Company filed a putative class action complaint in federal court in the Southern District of New York against the Company, Mr. Shkreli, Marc Panoff, and Jeffrey Paley (Kazanchyan v. Retrophin, Inc., Case No. 14-cv-8376). On December 16, 2014, a second, related complaint was filed in the Southern District of New York against the same defendants (Sandler v. Retrophin, Inc., Case No. 14-cv-9915). The complaints assert violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 in connection with defendants' public disclosures during the period from November 13, 2013 through September 30, 2014. In December 2014, plaintiff Kazanchyan filed a motion to appoint lead plaintiff, to approve lead counsel, and to consolidate the two related actions. On February 10, 2015, the Court consolidated the two actions, appointed lead plaintiff, and approved lead counsel. Lead plaintiff filed a consolidated amended complaint on March 4, 2015, which again named the Company, Mr. Shkreli, Mr. Panoff, and Mr. Paley as defendants, but which also named Steven Richardson, Stephen Aselage, and Cornelius Golding as additional defendants. On May 26, 2015, with the consent of the lead plaintiff, the court ordered that the claims against Mr. Paley be dismissed. The remaining defendants, including the Company, filed motions to dismiss the consolidated amended complaint, which were fully-briefed as of October 29, 2015. On December 1, 2015, counsel jointly informed the Court that the parties had reached a comprehensive settlement, subject to Court approval. On January 29, 2016, the parties filed motion for preliminary approval of the settlement and supporting papers, including a stipulation of settlement. On February 2, 2016, the Court preliminarily approved the settlement and scheduled a final approval hearing for June 10, 2016. Any amounts owed by the Company would be covered by Director and Officer Insurance.

In January 2015, the Company received a subpoena relating to a criminal investigation by the U.S. Attorney for the Eastern District of New York. The subpoena requested information regarding, among other things, the Company's relationship with Mr. Shkreli and individuals or entities that had been investors in investment funds previously managed by Mr. Shkreli. The Company has been informed that it is not a target of the U.S. Attorney's investigation, and is cooperating with the investigation. On December 17, 2015, an indictment against the Company's former Chief Executive Officer, Martin Shkreli, and its former outside counsel, Evan Greebel, was unsealed in the United States District Court for the Eastern District of New York. The Company has also been cooperating with a parallel investigation by the U.S. Securities and Exchange Commission (the "SEC"). On December 17, 2015, the SEC filed a civil complaint against Mr. Shkreli, Mr. Greebel, MSMB Capital Management LLC, and MSMB Healthcare Management LLC in the United States District Court for the Eastern District of New York.

On August 17, 2015, the Company filed a lawsuit in federal district court for the Southern District of New York against Martin 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 (Retrophin, Inc. v. Shkreli, 15-CV-06451(NRB)). On August 19, 2015, 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 has asserted counterclaims in the arbitration that are substantially similar to the claims it previously asserted in the federal lawsuit against Mr. Shkreli. The parties have selected an arbitration panel. On Mr. Shkreli's application, and with the Company's consent, the federal Court has granted a stay of the federal lawsuit pending a determination by the arbitration panel whether the Company's counterclaims will be litigated in the arbitration, as the Company is seeking. As of December 31, 2015 no accruals for loss contingencies have been recorded since the outcome of these cases are neither probable nor reasonably estimable. From time to time the Company is involved in legal proceedings arising in the ordinary course of business. The Company believes there is no other litigation pending that could have, individually or in the aggregate, a material adverse effect on its results of operations or financial condition.

Under the Company's bylaws, current and former officers and directors may seek advancement for certain expenses, including attorneys' fees. The Company has recently received a number of significant requests for advancement, and is in discussions about how much of the amounts sought the Company is obligated to pay. In addition, for certain of these amounts, the Company's obligation to pay advancement is eligible for reimbursement under the Company's insurance policies. Therefore, the Company is unable at this time to estimate the amount of advancement currently sought from the Company that will ultimately be eligible for advancement, nor how much of the amounts eligible for advancement will be eligible for reimbursement under the Company's insurance policies, and whether or not the amounts could be material.



ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

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## PART II

## Item 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

## Market Information

Our common stock is listed for quotation on the NASDAQ Global Market under the trading symbol "RTRX" and is part of the NASDAQ Biotechnology Index (NASDAQ: NBI).

As of February 24, 2016, the last reported sale price of our Common Stock as reported by the NASDAQ was \$14.98. The following table sets forth the high and low sales prices for our common stock for each full quarterly period within the two most recent fiscal years as reported by the NASDAQ.

Quarter Ending	High	Low
Fiscal Year 2015		
First Quarter	\$24.71	\$11.87
Second Quarter	\$34.68	\$21.12
Third Quarter	\$37.04	\$18.34
Fourth Quarter	\$23.04	\$17.20
Fiscal Year 2014		
First Quarter	\$21.84	\$7.19
Second Quarter	\$24.25	\$10.17
Third Quarter	\$14.49	\$8.85
Fourth Quarter	\$14.36	\$7.85

As of February 24, 2016, we had approximately 215 holders of record of our common stock.

## Performance Graph

The following is not deemed "filed" with the Securities and Exchange Commission and is not to be incorporated by reference into any filing we make under the Securities Act of 1933, as amended, whether made before or after the date hereof and irrespective of any general incorporation by reference language in such filing.

Our common stock is traded on the NASDAQ Global Market and is a component of both the NASDAQ Composite Index and the NASDAQ Biotechnology Index. The total return for our common stock and for each index assumes the reinvestment of dividends, although dividends have never been declared on our common stock, and is based on the returns of the component companies weighted according to their capitalizations as of the end of each monthly period. The NASDAQ-Composite tracks the aggregate price performance of equity securities of companies traded on the NASDAQ National Market. The NASDAQ Biotechnology Index contains securities and tracks the aggregate price performance of equity securities of NASDAQ-listed companies classified according to the Industry Classification Benchmark as either Biotechnology or Pharmaceuticals which also meet other eligibility criteria. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.

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Dividends

Since inception we have not paid any dividends on our common stock. We currently do not anticipate paying any cash dividends in the foreseeable future on our common stock. Although we intend to retain our earnings, if any, to finance the exploration and growth of our business, our Board of Directors will have the discretion to declare and pay dividends in the future. Payment of dividends in the future will depend upon our earnings, capital requirements and other factors which our Board of Directors may deem relevant.

Item 6. Selected Financial Data

The following table presents selected historical financial data of the Company for the periods indicated. The selected historical financial information is derived from the audited consolidated financial statements of the Company referred to under Item 8 of this Annual Report on Form 10-K, and previously published historical financial statements. The following selected financial data should be read in conjunction with Item 7 - Management's Discussion and Analysis of Financial Condition and Results of Operations, and the Company's Consolidated Financial Statements, including the notes thereto, included elsewhere herein.

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Consolidated Statement of Operations:	December 31, 2015	December 31, 2014	December 31, 2013
Net product sales	\$99,892	\$28,203	\$—
Total operating expenses	150,640	108,011	24,773
Operating loss	(50,748 )	(79,808 )	(24,773 )
Total other income (expenses), net	156,215	(33,590 )	(9,776 )
Income (Loss) before benefit (provision) for income taxes	105,467	(113,398 )	(34,549 )
Income tax benefit (provision)	11,770	2,460	(76 )
Net income (loss)	\$117,237	\$(110,938 )	\$(34,625 )
Per Share Data:			
Net Income (loss) per common share, basic	\$3.49	\$(4.43 )	\$(2.44 )
Net Income (loss) per common share, diluted	\$3.17	\$(4.43 )	\$(2.44 )
Weighted average common shares outstanding, basic	33,560,249	25,057,509	14,205,264
Weighted average common shares outstanding, diluted	37,581,439	25,057,509	14,205,264
Balance Sheet data:			
	December 31, 2015	December 31, 2014	December 31, 2013
Cash, cash equivalents and marketable securities	\$229,604	\$27,760	\$6,130
Working capital (deficit)	216,134	(70,205 )	(29,064 )
Total assets	512,400	135,471	20,499
Long-term debt	43,902	43,288	—
Total stockholders' equity (deficit)	\$299,971	\$(37,251 )	\$(19,667 )

Note: Cash dividends were not paid during the above periods.

The year ended December 31, 2013 information has been restated for the following:

In January 2015, our board of directors appointed an Oversight Committee of the board of directors (the "Oversight Committee"). The Oversight Committee concluded that certain transactions were consummated without specific approval of our board of directors or without our board of directors knowing all of the relevant facts. As a result, the financial statements contained in the Company's Form 10-Q for the three months ended September 30, 2013 (the "2013 Q3 Form 10-Q"), the Company's Form 10-K for the year ended December 31, 2013 (the "2013 Form 10-K") and the Company's Forms 10-Q for the quarters ended March 31, 2014, June 30, 2014 and September 30, 2014 (the "2014 Forms 10-Q") contained errors related to certain of the consulting agreements, the predominant purpose of which appears to have been to settle and release claims against investment funds previously managed by the former Chief Executive Officer of Retrophin, personally. On February 19, 2015, our board of directors concluded that as a result of the errors related to such consulting agreements, the financial statements contained in the 2013 Q3 Form 10-Q and the 2013 Form 10-K should no longer be relied upon. The Quarterly Report on Form 10-Q for the quarter ending September 30, 2013 and the Annual Report on Form 10-K for the year ended December 31, 2013 were amended and filed with the SEC in July 2015.

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### Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion should be read in conjunction with our audited consolidated financial statements, including the notes thereto.

#### Overview

#### Business and Recent Developments

We are a fully integrated biopharmaceutical company with approximately 130 employees headquartered in San Diego, California, focused on the development, acquisition and commercialization of therapies for the treatment of serious, catastrophic or rare diseases.

During the first quarter of 2015, the Company completed the acquisition of all worldwide rights, titles, and ownership of Cholbam (cholic acid), the first FDA approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for patients with peroxisomal disorders (including Zellweger spectrum disorders). The Company generated the first sales from Cholbam (known as Kolbam in the European Union) in April 2015.

We currently sell the following three products:

Chenodal (chenodeoxycholic acid) is approved in the United States for the treatment of patients suffering from gallstones in whom surgery poses an unacceptable health risk due to disease or advanced age. Chenodal has been the standard of care for cerebrotendinous xanthomatosis ("CTX") patients for more than three decades and the Company is currently pursuing adding this indication to the label.

Cholbam (cholic acid) is approved in the United States for the treatment of bile acid synthesis disorders due to single enzyme defects and is further indicated for adjunctive treatment of patients with peroxisomal disorders.

Thiola (tiopronin) is approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.

#### Divestiture of Assets:

##### Sale of Assets to Sanofi

The FDA granted Asklepiion Pharmaceuticals, LLC ("Asklepiion") a Rare Pediatric Disease Priority Review Voucher ("Pediatric PRV"), awarded to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A Pediatric PRV is transferable and provides the bearer with FDA priority review classification for a new drug application. The Pediatric PRV was transferred to Retrophin under the terms of the asset purchase agreement between the Company and Asklepiion dated January 12, 2015, pursuant to which the Company acquired Cholbam.

On July 2, 2015, the Company sold and transferred the Pediatric PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million is due on each of the first and second anniversaries of the closing. In accordance with accounting principles generally accepted in the United States ("U.S. GAAP"), the Company recorded the future short term and long term receivables at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale. The gain from the sale of the asset was approximately \$140.0 million, net of \$4.9 million in fees contractually due as part of the Cholbam acquisition.

##### Sale of Assets to Turing

On October 13, 2014, the Company entered into a binding Summary Separation Proposal with its then-current Chief Executive Officer. Among other things, the Summary Separation Proposal set forth a summary of the terms for the sale of the Company's Vecamyl, Syntocinon and ketamine licenses and assets to Turing Pharmaceuticals, a company controlled by the former Chief Executive Officer.

On January 9, 2015, the Company entered into a purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals the Sold Assets for a purchase price of \$1.0 million, and pursuant to which Turing Pharmaceuticals also assumed all future liabilities related to the Sold Assets.

On February 13, 2015, the Sellers entered into a purchase agreement with Waldun, pursuant to which the Sellers sold Waldun the Vecamyl Product Rights for a purchase price of \$0.7 million. Waldun in turn sold the Vecamyl Product Rights to Turing Pharmaceuticals. In connection therewith, on February 13, 2015, the Company, together with Manchester, entered into an asset purchase agreement with Turing Pharmaceuticals, pursuant to which the Company sold Turing Pharmaceuticals the Inventory for a purchase price of \$0.3 million, and pursuant to which Turing

Pharmaceuticals also assumed certain liabilities related to the Vecamyl Product Rights and the Inventory.

On February 13, 2015, the Company entered into an asset purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals its Oxytocin Assets, including related inventory, for a purchase price of \$1.1 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Oxytocin Assets.

The total impact to the Statement of Operations and Comprehensive Income (Loss) related to the divestitures for 2015 was \$0.9 million. See Note 9. to the financial statements for more information.

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2015 Public Offering

On March 24, 2015, the Company completed a public offering of 7,866,000 shares of common stock at a price of \$19.00 per share. The Company received net proceeds from the offering of \$140.0 million, after deducting underwriting fees and other offering costs of \$9.5 million. The shares of common stock were offered by the Company pursuant to a shelf registration statement that was declared effective by the SEC on March 13, 2015.

Kolbam in Europe

Kolbam, the bottled and branded name of Cholbam in Europe, was approved in the European Union ("EU") in April 2015. In June 2015, the General Court of the EU annulled the marketing authorization ("MA") for Kolbam due to a labeling conflict with the competitor product Orphacol. The EU Commission approved a new MA with revised labeling on November 20, 2015.

Products and Research and Development Programs

Chenodal (chenodiol tablets)

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

On March 26, 2014, we completed the acquisition of Manchester Pharmaceuticals including the U.S. rights for Chenodal and the intellectual property to develop, manufacture, and sell the product in the United States.

We are exploring the steps necessary to gain U.S. Food and Drug Administration ("FDA") approval of Chenodal for the treatment of CTX, a rare autosomal recessive lipid storage disease for which there are no FDA approved treatments.

We are exploring options related to the development of Chenodal for other indications.

Cholbam (cholic acid)

On March 18, 2015, the Company announced that the FDA approved Cholbam capsules, 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. Approximately 30 patients have transitioned from the open label extension trial to commercial product, and new patients have begun treatment. 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 have built a sales force to promote Thiola to targeted physicians.

Sparsentan

Sparsentan, also known as RE-021, is an investigational therapeutic agent which acts as both a potent angiotensin receptor blocker ("ARB"), as well as a selective endothelin receptor antagonist ("ERA"), with selectivity toward endothelin receptor type A. We have secured a license to sparsentan from Ligand and Bristol-Myers Squibb (who referred to it as DARA). We are developing sparsentan as a treatment for FSGS, which is a leading cause of end-stage renal disease. We are currently enrolling patients for the DUET Phase 2 clinical study of sparsentan for the treatment of FSGS and we anticipate having a top line data read out in the third quarter of 2016. Depending on the data obtained in the DUET study, we may be able to support an application for accelerated approval for sparsentan on the basis of proteinuria as a surrogate endpoint. Sparsentan was granted fast track designation in June 2015 and orphan drug designation in the U.S. and EU in January and November 2015, respectively.

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### RE-024

We are developing 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. PKAN is estimated to affect 1 to 3 persons per million. There are currently no viable treatment options for patients with PKAN. RE-024 is a phosphopantothenate prodrug therapy that aims to restore levels of this key substrate in PKAN patients. Certain international health regulators have approved the initiation of dosing RE-024 in PKAN patients under physician-initiated studies in accordance with local regulations in their respective countries. The Company filed a U.S. IND for RE-024 with the FDA in the first quarter of 2015 to support the commencement of a Company-sponsored Phase 1 study, which initiated in April 2015. RE-024 was granted orphan drug designation from the FDA in May 2015 and was granted fast track designation in June 2015.

### RE-034 (Tetracosactide Zinc)

RE-034 is a synthetic hormone analog of the first 24 amino acids of the 39 amino acids contained in adrenocorticotrophic hormone (“ACTH”) incorporated into a novel formulation developed by the Company. RE-034 exhibits the same physiological actions as endogenous ACTH by binding to all five melanocortin receptors (pan-MCR), resulting in its anti-inflammatory and immunomodulatory effects. The Company has successfully formulated and manufactured RE-034 at proof-of-concept scale using a novel formulation process that allows modulation of the release of the active ingredient from the site of administration. The Company intends to continue preclinical development of RE-034 to enable multiple strategic options.

### Financial Overview

#### Compensation and Related Costs

Compensation and related costs include salaries, bonuses, non-cash share based equity compensation and benefits to our executives and employees.

#### Professional Fees

Professional fees include: legal expenses related to licensing, product acquisition and investigation, employment and consulting agreements, general corporate work, consulting fees, accounting fees, and public and investor relations fees.

#### Research and Development Costs

Research and development include expenses related to RE-021, RE-034 and RE-024 and our other pipeline programs. We expense all research and development costs as they are incurred. Our research and development costs are comprised of salaries and bonuses, benefits, non-cash share based compensation, license fees, milestones under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials and delivery devices, and associated overhead expenses and facilities costs. Reimbursed research and development costs under collaborative arrangements are recorded as a reduction to research and development costs. We charge direct internal and external program costs to the respective development programs. We also incur indirect costs that are not allocated to specific programs because such costs benefit multiple development programs and allow us to increase our pharmaceutical development capabilities. These consist primarily of facilities costs and other internal shared resources related to the development and maintenance of systems and processes applicable to all of our programs.

At any point in time, we typically have various early stage research and drug discovery projects. Our internal resources, employees and infrastructure are not directly tied to any one research or drug discovery project and are typically deployed across multiple projects. As such, we do not maintain information regarding these costs incurred for these early stage research and drug discovery programs on a project-specific basis.

We routinely engage vendors and service providers for scientific research, clinical trial, regulatory compliance, manufacturing and other consulting services. We also make grants to research and non-profit organizations to conduct research which may lead to new intellectual properties that we may subsequently license under separately negotiated license agreements. Such grants may be funded in lump sums or installments.

The following table summarizes our research and development expenses during the years ended December 31, 2015, 2014 and 2013. The internal costs include personnel, facility costs, laboratory consumables and discovery and



research related activities associated with our pipeline. The external program costs reflect external costs attributable to our clinical development candidates and preclinical candidates selected for further development.

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Such expenses primarily include third-party contract costs relating to manufacturing, clinical trial activities, translational medicine and toxicology activities.

	For the Year Ended December 31,		
	2015	2014	2013
External service provider costs:		(in thousands)	
Sparsentan	\$11,179	\$7,449	\$2,443
RE-024	7,631	11,175	1,549
Syntocinon	—	3,353	251
RE-034	357	3,237	230
General	6,754	7,077	159
Other product candidates	696	1,829	1,118
Total external service provider costs:	26,617	34,120	5,750
Internal personnel costs:	23,809	13,675	1,334
Total research and development	\$50,426	\$47,795	\$7,084

We expect our research and development expenses to increase during fiscal 2016 as we focus on clinical trials for our key product candidates, advance our discovery research projects into the preclinical stage and continue our early stage research. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time consuming. We may never succeed in achieving marketing approval for any of our product candidates. The probability of success of each product candidate may be affected by numerous factors, including preclinical data, clinical data, competition, manufacturing capability and commercial viability.

Most of our product development programs are in clinical trials which are highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each product candidate and are difficult to project. Given the uncertainty associated with clinical trial enrollments and the risks inherent in the development process, we are unable to determine the duration and completion costs of current or future clinical trials of our product candidates or if and to what extent we will generate revenues, if any, from the commercialization and sale of any of our product candidates.

**Selling, General and Administrative**

Selling, general and administrative expenses consist of salaries and bonuses, benefits, non-cash share based compensation, professional fees, rent, depreciation and amortization, travel and entertainment, recruiting, insurance, business development, advertising, and other operating expenses.

**Other Expenses**

Other expenses consist of the change in fair value of derivative financial instruments, litigation settlement gain, interest income and expense, finance expense, bargain purchase gain, loss on the extinguishment of debt, debt early payment penalty, and gain on sale of assets.

**License Agreements****Ligand**

We have a worldwide license from Ligand for the development, manufacture and commercialization of sparsentan, an ARB and ERA which we are initially developing in connection with the treatment of FSGS. Under the license agreement, Ligand granted us a sublicense under certain of its patents and other intellectual property in connection with the development and commercialization of sparsentan. Under the license agreement, Ligand is obligated to transfer to us certain information, records, regulatory filings, materials and inventory controlled by Ligand and relating to or useful for developing sparsentan. We must use commercially reasonable efforts to develop and commercialize sparsentan in specified major market countries and other countries in which we believe it is commercially reasonable to develop and commercialize such products.

As consideration for the license, we are required to make payments payable upon the achievement of certain milestones totaling up to \$109.4 million. Should we commercialize sparsentan or any products containing any of these compounds, we will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of

all such products. Through December 31, 2015 and 2014, we made payments to Ligand of \$3.8 million and \$2.5 million, respectively, under the license agreement.

Under the terms of the license agreement, Bristol-Myers Squibb will have a right of first negotiation and Ligand will have a right of second negotiation with respect to any license arrangement for a licensed compound.

The license agreement will continue until neither party has any further payment obligations under the agreement and is expected to continue for approximately 10 to 20 years. Ligand may also terminate the license agreement due to (i) our insolvency, (ii) our material uncured breach of the agreement, (iii) our failure to use commercially reasonable efforts to develop and commercialize sparsentan as described above or (iv) certain other conditions. We may terminate the license agreement due to a material uncured breach of the agreement by Ligand.

Thiola License Agreement

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On May 29, 2014, the Company entered into a license agreement with Mission Pharmacal Company ("Mission"), pursuant to which Mission agreed to grant the Company an exclusive, royalty-bearing license to market, sell and commercialize Thiola (Tiopronin) in the United States and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola. In July 2014, the Company amended the license agreement with Mission to secure the Canadian marketing rights to the product for no additional consideration.

Upon execution of the agreement, the Company paid Mission an up-front license fee of \$3 million. In addition, the Company shall pay guaranteed minimum royalties during each calendar year the greater of \$2 million or twenty percent (20%) of the Company's net sales of Thiola through June 30, 2024. As of December 31, 2015, the present value of guaranteed minimum royalties payable is \$10.9 million using a discount rate of approximately 11% based on the Company's current borrowing rate. As of December 31, 2015, the guaranteed minimum royalties' current and long term liability is approximately \$0.8 million and \$10.1 million, respectively, and is recorded as guaranteed minimum royalty in the consolidated balance sheet. The Company capitalized \$24.1 million related to the Thiola asset which consists of the up-front license fee, professional fees, present value of the guaranteed minimum royalties and any additional payments through 2015 in excess of minimum royalties.

**Other Matters****Investigation and Impact on Financial Statements**

In September 2014, the Company's board of directors requested that its outside legal counsel conduct an investigation into various matters related to the former Chief Executive Officer of the Company. In January 2015, our board of directors appointed an Oversight Committee to oversee and direct the investigation and make findings and decisions related to the investigation. As a result of the investigation, the Oversight Committee determined that, throughout 2013 and 2014, the former Chief Executive Officer engaged in a series of transactions (the "Prior Transactions"), which involved individuals and entities that had been investors in investment funds previously managed by the former Chief Executive Officer (the "MSMB Entities"), pursuant to which assets of the Company were misappropriated.

In addition, as a result of the Prior Transactions the financial statements contained in the Company's Form 10-Q for the three months ended September 30, 2013 (the "2013 Q3 Form 10-Q"), the Company's Form 10-K for the year ended December 31, 2013 (the "2013 Form 10-K") and the Company's Forms 10-Q for the quarters ended March 31, 2014, June 30, 2014 and September 30, 2014 (the "2014 Forms 10-Q") contained errors related to the reporting of certain consulting agreements entered into as part of the Prior Transactions, the predominant purpose of which appears to have been to settle and release claims against the MSMB Entities or the former Chief Executive Officer personally. On February 19, 2015, our board of directors concluded that as a result of the errors related to such consulting agreements, the financial statements contained in the 2013 Q3 Form 10-Q and the 2013 Form 10-K should no longer be relied upon. The Quarterly Report on Form 10-Q for the quarter ending September 30, 2013 and the Annual Report on Form 10-K for the year ended December 31, 2013 were amended and filed with the SEC in July 2015.

**Stock Option Accounting**

We held a Special Meeting of Stockholders on February 3, 2015, at which our stockholders voted to approve a proposal ratifying the prior issuance of stock options to purchase 1,928,000 shares of common stock and 230,000 restricted shares of common stock granted to employees between February 24, 2014 and August 18, 2014 (the "Ratified Equity Grants"). The 2014 Forms 10-Q contained errors related to the non-cash compensation expense recognized in connection with the Ratified Equity Grants, because the grant/measurement date of the Ratified Equity Grants for financial accounting purposes did not occur until their ratification.

We previously accounted for the Ratified Equity Awards as if a grant/measurement date for financial accounting purposes had occurred upon their issuance date, and recognized compensation expense for such Ratified Equity Awards based on the grant/measurement date value, which is amortized ratably to compensation expense and additional paid-in capital over the applicable service periods. We should have accounted for the Ratified Equity Awards as equity grants without a grant/measurement date, which are accounted for as "liability awards", with compensation expense and an offsetting compensation liability recorded over the term of the award, and the liability award revalued at each reporting period based on changes in the Company's stock price until it is ratified.

We believe that the errors in the 2014 Forms 10-Q related to the non-cash compensation expense recognized in connection with the Ratified Equity Grants, do not cause the financial statements within the 2014 Forms 10-Q to be

misleading, and therefore such financial statements can still be relied upon. The Company restated those quarters in 2015 Form 10-Q filings.

On February 27, 2015, the Company received a Public Letter of Reprimand from NASDAQ (“Letter of Reprimand”), in accordance with Nasdaq Listing Rule 5810(c)(4). The Letter of Reprimand communicates NASDAQ’s belief that the interests of the Company’s shareholders were not materially adversely affected by the matters described above, and while not having been cured, the violation described above was remediated to the extent possible. Accordingly, NASDAQ does not believe that the delisting of the Company’s securities is an appropriate sanction, but rather, the circumstances warranted the issuance of the Letter of Reprimand. The issuance of the Letter of Reprimand completes NASDAQ’s review of the matters described above.

Results of Operations for the Years Ended December 31, 2015, 2014 and 2013

Net Product Sales

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

	Year Ended December 31,		
	2015	2014	2013
Net product sales	\$99,892	\$28,203	\$—

Net product sales for the years ended December 31, 2015, 2014 and 2013 were \$99.9 million, \$28.2 million and \$0 million, respectively, and consisted of sales of Thiola, Vecamyl, Chenodal and Cholbam in 2015, and sales of Thiola, Chenodal and Vecamyl in 2014, less allowances for government rebates and patient assistance programs. Retrophin had no product sales in 2013.

The increase in net product sales for the year ended December 31, 2015 as compared to the same period in 2014, is due to a full year of sales for Chenodal and Thiola, increased growth of Thiola sales due to new patient starts, and the addition of Cholbam to our product group in April 2015.

The increase in net product sales for the year ended December 31, 2014 as compared to the same period in 2013, primarily reflects generation of our first sale in March 2014 after completing the acquisition of all of the membership interests of Manchester on March 26, 2014. In May 2014 we entered into a license agreement with Mission for the U.S. marketing rights to Thiola.

The Company uses a direct-to-patient distributor. Under this distribution model, the Company records revenues when the distributor ships products to customers and such customers take title of the product.

#### Operating Expenses:

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

	Year Ended December 31,			Year Ended December 31,		
	2015	2014	Increase	2014	2013	Increase
Cost of goods sold	\$2,185	\$571	\$1,614	\$571	\$—	\$571
Research and development	50,426	47,795	2,631	47,795	7,084	40,711
Selling, general and administrative	79,541	59,645	19,896	59,645	17,689	41,956
Change in valuation of contingent consideration	13,778	—	13,778	—	—	—
Impairment of intangible assets	4,710	—	4,710	—	—	—
	\$150,640	\$108,011	\$42,629	\$108,011	\$24,773	\$83,238

#### 2015 versus 2014 results

Our operating expenses for the year ended December 31, 2015, were \$150.6 million compared to \$108.0 million for the year ended December 31, 2014, an increase of \$42.6 million. The operating expenses increase is attributable to an increase in our research and development expenses of \$2.6 million, an increase in selling, general and administrative expenses of \$19.9 million, a change in valuation of contingent consideration of \$13.8 million, and impairment of intangible assets of \$4.7 million.

The increase in research and development costs of \$2.6 million is primarily due to an increase of \$10.1 million from headcount and compensation related to the hiring of critical regulatory and development expertise. This was offset by divestiture of Syntocinon, the timing of RE-024 preclinical studies, and lower spending on RE-034.

The increase in selling, general and administrative expenses of \$19.9 million is primarily due to an increase in sales and marketing personnel and associated activities of \$6.4 million to support our commercialization efforts in the current year, stock compensation increases of \$5.5 million and amortization from intangible assets of \$8.0 million. In addition, the Company incurred charges of \$13.8 million and \$4.7 million in operating expenses related to the revaluation of contingent consideration liabilities for the products Chenodal and Cholbam, and the write-off of intangible assets related to Carbetocin as the Company elected not to pursue internal development of the asset, respectively.

#### 2014 versus 2013 results

Our operating expenses for the year ended December 31, 2014 were \$108.0 million compared to \$24.8 million for the year ended December 31, 2013, an increase of \$83.2 million. The operating expenses increase is attributable to an increase in our research and development expenses of \$40.7 million and an increase in selling general and administrative expenses of \$42.0 million. The growth in the Company's overall operating expenses is due to the

Company becoming a commercial company and supporting the launch of three revenue producing drugs during fiscal 2014. During this time, the number of Retrophin employees increased from 27 as of the end of fiscal 2013 to 110 at the end of fiscal 2014.

The increase in research and development costs of \$40.7 million is due to an increase in research and development expenses for RE-024 of \$9.6 million and an increase of \$5.0 million in research and development expenses for sparsentan as we moved these assets through development. In addition, an increase of \$12.3 million of research and development personnel costs to support our pipeline drugs contributed to the overall period increase.

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The increase in selling, general and administrative expenses of \$42.0 million is due to an overall increase in compensation expense of \$18.8 million attributable to an increase in the number of Retrophin employees during the year, in addition to an increase of \$12.7 million in legal, professional, and accounting fees. Other selling, general and administrative costs increased \$10.2 million due to an increase of \$1.3 million in business development activities, an increase of \$4.6 million related to amortization of intangible assets and an increase in sales and marketing expenses of \$2.9 million.

## Other Income/Expenses:

The following table provides information regarding Other Income (Expenses) (in thousands):

	Year Ended December 31,			Year Ended December 31,		
	2015	2014	Variance	2014	2013	Variance
Litigation settlement gain	\$15,500	\$—	\$15,500	\$—	\$—	\$—
Other income (expense), net	(296	) 2,352	(2,648	) 2,352	370	1,982
Interest expense, net	(7,748	) (7,435	) (313	) (7,435	) (46	) (7,389
Debt early payment penalty	(2,250	) —	(2,250	) —	—	—
Loss on extinguishment of debt	(4,151	) —	(4,151	) —	—	—
Finance expense	(600	) (4,721	) 4,121	(4,721	) —	(4,721
Change in fair value of derivative instruments	(33,307	) (23,786	) (9,521	) (23,786	) (10,100	) (13,686
Gain on sale of assets	140,004	—	140,004	—	—	—
Bargain purchase gain	49,063	—	49,063	—	—	—
	\$156,215	\$(33,590	) \$189,805	\$(33,590	) \$(9,776	) \$(23,814

Other income for the year ended December 31, 2015 was \$156.2 million compared to other expense of \$33.6 million for the year ended December 31, 2014, which represents an increase of \$189.8 million. The increase was primarily attributable to the gain on the sale of the Pediatric PRV to Sanofi, the bargain purchase gain on the Cholbam acquisition and the litigation settlement gain, offset by the change in fair value of derivative instruments, driven by the increase in the Company's stock price.

Other expenses for the year ended December 31, 2014 was \$33.6 million compared to \$9.8 million for the year ended December 31, 2013 which represents an increase of \$23.8 million. The other expense increase was primarily attributable to charges due to the change in the fair value of derivative financial instruments of \$13.7 million, an increase in interest expense of \$7.4 million as the Company entered into a \$46 million convertible notes agreement and a \$45 million Credit Agreement during fiscal 2014 and an increase in finance expense of \$4.7 million, offset by an increase in the realized gains on the sale of marketable securities of \$2.0 million.

## Income Tax Benefit (Provision):

The Company follows ASC 740, Income Taxes, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are based on the differences between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance to the extent management concludes it is more likely than not that the asset will not be realized.

The standard addresses the determination of whether tax benefits claimed or expected to be claimed on a tax return should be recorded in the financial statements. Under ASC 740, the Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the tax authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position should be measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement. ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. The Company's policy is to record estimated interest and penalty related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision.



Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements, except for operating leases.

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 in the near term. 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.

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For the years ended December 31, 2015 and 2014 the Company had the following financial performance (in thousands):

	December 31, 2015	December 31, 2014
Revenue	\$99,892	\$28,203
Net Income (Loss)	117,237	(110,938 )
Cash & Cash Equivalents	37,805	18,204
Short Term Investments	191,799	9,556
Accumulated Deficit	(65,153	) (179,175 )
Stockholders' Equity (Deficit)	299,971	(37,251 )
Net Working Capital (Deficit)	\$216,134	\$(70,205 )
Net Working Capital Ratio	3.52	0.35

## Equity Offering

In March 2015, we completed a public offering of 7,866,000 shares of common stock at a price of \$19.00 per share. We received net proceeds from the offering of \$140.0 million, after deducting underwriting fees and other offering costs of \$9.5 million.

On January 9, 2014, we completed a public offering of 4,705,882 shares of common stock at a price of \$8.50 per share. We received net proceeds from the offering of \$36.8 million, after deducting the underwriting fees and other offering costs of \$3.2 million.

## Sale of Assets

## Assets sold to Turing

On October 13, 2014, the Company entered into a binding Summary Separation Proposal with its then-current Chief Executive Officer. Among other things, the Summary Separation Proposal set forth a summary of the terms for the sale of the Company's Vecamyl, Syntocinon and ketamine licenses and assets to Turing Pharmaceuticals, a company controlled by the former Chief Executive Officer.

On January 9, 2015, the Company entered into a purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals the Sold Assets for a purchase price of \$1.0 million, and pursuant to which Turing Pharmaceuticals also assumed all future liabilities related to the Sold Assets.

On February 13, 2015, the Sellers entered into a purchase agreement with Waldun, pursuant to which the Sellers sold Waldun the Vecamyl Product Rights for a purchase price of \$0.7 million. Waldun in turn sold the Vecamyl Product Rights to Turing Pharmaceuticals. In connection therewith, on February 13, 2015, the Company, together with Manchester, entered into an asset purchase agreement with Turing Pharmaceuticals, pursuant to which the Company sold Turing Pharmaceuticals the Inventory for a purchase price of \$0.3 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Vecamyl Product Rights and the Inventory.

On February 13, 2015, the Company entered into an asset purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals its Oxytocin Assets, including related inventory, for a purchase price of \$1.1 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Oxytocin Assets.

The total impact to the Statement of Operations and Comprehensive Income (Loss) related to the divestitures for 2015 was \$0.9 million. See Note 9. to the financial statements for more information.

## Assets sold to Sanofi

On July 2, 2015, the Company sold and transferred the Pediatric PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million is due on each of the first and second anniversaries of the closing. In accordance with U.S. GAAP, the Company recorded the future short term and long term receivables at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale. The gain from the sale of the asset was approximately \$140.0 million, net of \$4.9 million in fees contractually due as part of the Cholbam acquisition.

## Borrowings

## Convertible Notes Payable

On May 29, 2014, the Company entered into the Note Purchase Agreement relating to a private placement by the Company of \$46.0 million aggregate principal senior convertible notes due 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, beginning on November 15. The Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with the terms. The aggregate carrying value of the Notes on their issuance was \$43.0 million, which was net of the \$3.0 million debt discount.

On June 30, 2014, the Company issued 401,047 shares of Common Stock to the holders of the Notes and such Noteholders granted the Company a release of certain claims they may have had in connection with the Company's sale of the Notes or certain statements made by the Company in connection with such sale due to the former Chief Executive Officer's violation of his lockup agreement. The Company recorded finance expense as

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other expense in the amount of \$4.7 million for the year ended December 31, 2014 based on the fair market value of the stock on the date of issuance in relation to the shares issued.

### Credit Facility

In June 2014, the Company entered into a \$45 million Credit Agreement (“Credit Facility”) which would have matured on June 30, 2018 and bore interest at an annual rate of (i) the Adjusted LIBOR Rate (as such term is defined in the Credit Facility) plus 10.00% or (ii) in certain circumstances, the Base Rate (as such term is defined in the Credit Agreement) plus 9.00%. The Credit Facility contained certain covenants, including those limiting the Company's and its subsidiaries' abilities to incur indebtedness, incur liens, sell or acquire assets or businesses, change the nature of their businesses, engage in transactions with related parties, make certain investments or pay dividends. In addition, the Credit Facility required the Company and its subsidiaries to meet certain financial quarterly requirements. Failure by the Company or its subsidiaries to comply with any of these covenants or financial tests could result in the acceleration of the loans under the Credit Facility.

On January 12, 2015, the Company entered into Amendment No. 3 (“Amendment No. 3”) to the Credit Agreement in which the Company obtained a commitment letter from Athyrium Capital Management, LLC and Perceptive Credit Opportunities Fund, LP (collectively, the “Lenders”), the Company’s existing lenders, providing a commitment for a senior secured incremental term loan under the Company’s existing term loan facility in an aggregate principal amount of \$30 million (the “Incremental Loan”), which could have been drawn down at the Company’s option to finance the acquisition of assets.

As consideration for the commitment letter for the Incremental Loan, the Company made a cash payment to the Lenders and issued the Lenders warrants initially exercisable to purchase up to an aggregate of 125,000 shares of the Company’s common stock. The Company recorded a charge of \$1.1 million in interest expense.

On July 1, 2015, the Company paid \$47.3 million as payment in full for all principal and accrued interest under the Credit Facility, which included \$45.0 million to pay off the principal balance, \$2.3 million in prepayment premiums for early payment penalty, and an immaterial amount of interest accrued through the settlement date, as required by the terms of the Credit Agreement. Upon receipt of this final payment, the liens and security interests granted pursuant to the Credit Agreement and the documents executed and delivered pursuant thereto or in connection therewith were automatically and irrevocably released and terminated.

Total interest expense, net, recognized for the years ended December 31, 2015, 2014 and 2013 was \$7.7 million, \$7.4 million and \$0.1 million, respectively.

### License Agreement Obligations

#### Ligand License

As consideration for the sparsentan license, we are required to make payments upon the achievement of certain milestones totaling up to \$109.4 million. Should we commercialize sparsentan or any products containing any related compounds, we will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of all such products. During 2015, we made payments to Ligand of \$3.8 million under the license agreement.

#### 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 the near term. This belief is based on many factors, however some factors are beyond our control. Factors affecting our 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;  
the potential acquisition or 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.

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## Cash Flows

The following table summarizes our cash flows for the periods set forth below:

	2015	2014	2013
Net cash used in operating activities	\$(554)	\$(45,850)	\$(17,589)
Net cash used in investing activities	(80,602)	(37,263)	(5,406)
Net cash provided by financing activities	100,767	95,320	28,981
Net increase in cash	19,611	12,207	5,986
Effect of exchange rate changes on cash	(10)	—	—
Cash & cash equivalents, beginning of period	18,204	5,997	11
Cash & cash equivalents, end of period	\$37,805	\$18,204	\$5,997

Management considers marketable securities to be available to fund current operations, therefore, they are classified as available for sale and included within current assets in our consolidated balance sheets. Therefore, cash on hand is considered to be approximately \$229.6 million.

## Cash Flows from Operating Activities

Operating activities used \$0.6 million during the year ended December 31, 2015 compared to \$45.9 million for the year ended December 31, 2014. The decrease of \$45.3 million was the result of an increase in revenue of \$71.7 million, offset by a decrease in operating assets of \$25.3 million.

Operating activities used \$45.8 million of cash during the year ended December 31, 2014 compared to \$17.6 million for the year ended December 31, 2013. The increase of \$28.2 million was the result of an increase in net loss of \$76.3 million, decrease in non-cash charges of \$37.2 million, and a net change in operating assets and liabilities of \$10.8 million.

## Cash Flows from Investing Activities

Cash used in investing activities for the year ended December 31, 2015 was \$80.6 million compared to \$37.3 million for the year ended December 31, 2014. The increase of \$43.3 million was primarily the result of cash used in the purchase of marketable securities of \$198.5 million in 2015, partially offset by cash received from the divestiture of assets of \$148.4 million.

Cash used in investing activities for the year ended December 31, 2014 was \$37.3 million compared to \$5.4 million for the year ended December 31, 2013. The increase of \$31.9 million was primarily the result of the cash paid for the Manchester acquisition of \$29.2 million.

## Cash Flows from Financing Activities

For the year ended December 31, 2015, cash provided by financing activities was \$100.8 million compared to \$95.3 million during the year ended December 31, 2014. The cash provided in 2015 was the result of the issuance of common stock of \$140.0 million, net of fees, offset by the cash used to pay down debt of \$45.0 million. The cash provided in 2014 was the result of the issuance of common stock of \$40.0 million, net of fees, and the net proceeds from the Credit Agreement of \$42.4 million and net proceeds from the Note Purchase agreement of \$43.0 million, offset by the pay down of the Manchester Note payable of \$31.3 million.

For the year ended December 31, 2014, cash provided by financing activities was \$95.3 million compared to \$29.0 million during the year ended December 31, 2013. The increase of \$66.3 million was primarily a result of an increase of the net proceeds from the Credit Agreement of \$42.4 million and net proceeds from the Note Purchase agreement of \$43.0 million.

## Contractual Commitments

The following table summarizes our principal contractual commitments, excluding open orders that support normal operations, as of December 31, 2015 (in thousands):

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating leases	\$2,671	\$1,026	\$1,645	\$—	\$—
Note payable	53,073	2,070	4,140	46,863	—
Sales support services	3,470	312	937	833	1,388
Product supply contracts	5,221	3,923	1,298	—	—

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Purchase order commitments	596	258	338	—	—
	\$65,031	\$7,589	\$8,358	\$47,696	\$1,388

The contractual commitments table above does not include provisions for ASC 740-10 the Accounting for Uncertainty in Income Taxes.

Critical Accounting Policies and Estimates

Management's discussion and analysis of our financial position and results of operations are based upon our Consolidated Financial Statements, which are included in Item 8 of this report and have been prepared in accordance with accounting principles generally accepted in the United States. The

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preparation of these financial statements requires management to make estimates, judgments, assumptions and valuations that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosures. While management believes that its estimates, judgments and assumptions are appropriate, significant differences in actual experience or significant changes in assumptions may materially affect our future results. Management believes the critical accounting policies and areas that require the most significant estimates, judgments, assumptions or valuations used in the preparation of our financial statements are those summarized below.

### Revenue Recognition

Product sales for the year ended December 31, 2015 consisted of sales of Chenodal, Vecamyl (divested in 2015), Cholbam and Thiola. Product sales for the year ended December 31, 2014 consisted of sales of Chenodal, Vecamyl and Thiola. Revenue from product sales is recognized when persuasive evidence of an arrangement exists, title to product and associated risk of loss have passed to the customer, the price is fixed or determinable, collection from the customer is reasonably assured, the Company has no further performance obligations, and returns can be reasonably estimated. The Company sells in the United States and Canada through a direct-to-patient distributor. Under this distribution model, the Company records revenues when the distributor ships products to customers and such customers take title of the product.

Revenue from product sales is recorded net of applicable provisions for rebates under government programs (including Medicaid), prompt pay discounts, and other sales-related deductions. We review our estimates of rebates and other applicable provisions each period and record any necessary adjustments in the current period.

### Deductions from Revenue

**Government Rebates and Chargebacks:** The Company estimates the rebates that we will be obligated to provide to government programs and deducts 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.

**Prompt Pay Discounts:** The Company offers discounts to certain customers for prompt payments. The Company estimates these discounts based on customer terms and historical trends. The Company accrues for the estimated 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, the Company offers its 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 so returns are immaterial.

### Research and Development Costs

Research and development costs are expensed as incurred and include: salaries, benefits, bonus, stock-based compensation, license fees, milestone payments due under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials and delivery devices, and associated overhead and facilities costs. Clinical trial costs are a significant component of research and development expenses and include costs associated with third-party contractors, and clinical research organizations ("CRO's"). Invoicing from third-party contractors for services performed can lag several months. We accrue the costs of services rendered in connection with third-party contractor activities based on our estimate of management fees, and costs associated with site monitoring and data management.

### Employee Stock-Based Compensation

The Company recognizes all employee share-based compensation as a cost in the financial statements.

Equity-classified awards principally related to stock options, restricted stock units ("RSUs") and performance stock units ("PSUs"), are measured at the grant date fair value of the award. The Company determines grant date fair value of stock option awards using the Black-Scholes option-pricing model. The fair value of RSUs are determined using the closing price of the Company's common stock on the grant date. For service based vesting grants, expense is recognized over the implicit service period based on the number of options or shares expected to ultimately vest. For PSUs, expense is recognized over the requisite period until the performance obligation is met, assuming that it is probable. No expense is recognized for PSUs until it is probable the vesting criteria will be satisfied. Forfeitures are estimated at the date of grant and revised when actual or expected forfeiture activity differs materially from original



estimates.

	Vesting Terms
Stock Options	1 to 3 years
Restricted Stock Units	1 to 3 years
Trade and Notes Receivable	
Trade Receivables, Net	

Trade accounts receivable are recorded net of allowances for prompt payment and doubtful accounts. Allowances for rebate discounts are included in other current liabilities in the consolidated balance sheets. Estimates for allowances for doubtful accounts are determined based on existing contractual obligations, historical payment patterns and individual customer circumstances. The allowance for doubtful accounts was \$0 and \$0.1 million at December 31, 2015 and 2014, respectively.

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## Notes Receivable

Notes receivable arose from the sale of the pediatric priority review voucher (the "PRV"). On July 2, 2015, the Company sold and transferred the PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million is due on each of the first and second anniversaries of the closing. In accordance with accounting principles generally accepted in the United States ("U.S. GAAP"), the Company recorded the future short term and long term receivables at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale using a discount rate of 2.8%. The accretion on the notes receivable totaled \$1.3 million and is recorded in interest expense, net, in 2015. As of December 31, 2015, the present value of the notes receivable was \$46.8 million and \$45.6 million, respectively. The Company noted no indications for impairment as of December 31, 2015.

## Inventories and Related Reserves

Inventory is stated at the lower of cost or market. The Company determines the cost of inventory using the first-in, first-out, or FIFO, method. The Company periodically analyzes its inventory levels to identify inventory that may expire prior to expected sale or has a cost basis in excess of its estimated realizable value, and writes down such inventory as appropriate. In addition, the Company's products are subject to strict quality control and monitoring which the Company's manufacturers perform throughout their manufacturing process. The Company does not directly manufacture any product. The Company has single suppliers for products Chenodal and Thiola, and prospectively arranges for manufacture from contract service providers for its product Cholbam. The value of inventory acquired in 2015 related to single supplier purchases was 63% for Thiola and 4% for Chenodal. The remaining 33% of inventory was related to the Cholbam product and was either related to materials acquired or subsequent third party manufacturing. The inventory reserve was \$0.3 million and \$0.1 at December 31, 2015 and 2014, respectively.

Inventory, net of reserve, consists of the following at December 31, 2015 and 2014 (in thousands):

	December 31, 2015	December 31, 2014
Raw material	\$289	\$315
Finished goods	2,247	486
Total inventory	\$2,536	\$801

## Property and Equipment, net

Property, plant and equipment are stated at cost, net of accumulated depreciation. Depreciation is computed using the straight-line method over the related estimated useful lives as presented in the table below. Significant additions and improvements are capitalized, while repairs and maintenance are charged to expense as incurred. Property and equipment purchased for specific research and development projects with no alternative uses is expensed as incurred. The major classifications of property and equipment, including their respective expected useful lives, consists of the following:

Computer equipment	3 years
Furniture and fixtures	7 years
Leasehold improvements	Shorter of length of lease or life of the asset

## Intangible Assets, Net

Intangible assets with finite useful lives consist primarily of product rights, licenses and customer relationships which are amortized on a straight line basis over 1 to 16 years. Intangible assets with finite useful lives are reviewed for impairment and the useful lives are reassessed whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. If circumstances require a long-lived asset or asset group be tested for possible impairment, the Company first compares undiscounted cash flows expected to be generated by that asset or asset group to its carrying value. If the carrying value of the long-lived asset or asset group is not recoverable on an undiscounted cash flow basis, an impairment is recognized to the extent that the carrying value exceeds its fair value. The Company reviews for indications of impairment of intangibles on a quarterly basis.

For the year ended December 31, 2015 the company wrote off the intangible asset related to Carbetocin and recorded a loss of \$4.7 million. There were no impairments related to intangible assets for 2014 or 2013.

## Income Taxes

The Company follows ASC 740, Income Taxes, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are based on the differences between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance to the extent management concludes it is more likely than not that the asset will not be realized.

The standard addresses the determination of whether tax benefits claimed or expected to be claimed on a tax return should be recorded in the financial statements. Under ASC 740, the Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the tax authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position should be measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon

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ultimate settlement. ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. The Company's policy is to record estimated interest and penalty related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision.

### Patents

The Company expenses external costs, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company also expenses costs associated with maintaining and defending patents subsequent to their issuance in the period incurred.

### Derivative Instruments

The Company does not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. The Company evaluates all of its financial instruments to determine if such instruments are derivatives or contain features that qualify as embedded derivatives. For derivative financial instruments that are accounted for as liabilities, the derivative instrument is initially recorded at its fair value and is then revalued at each reporting date, with changes in the fair value reported in the statements of operations. For stock-based derivative financial instruments, the Company calculates the fair value of the financial instruments using the Monte Carlo simulation pricing model, however, prior to January 1, 2015 the Company used the Binomial Lattice option pricing model. The classification of derivative instruments, including whether such instruments should be recorded as liabilities or as equity is assessed at inception, the fair value of the warrants is evaluated at the end of each reporting period.

### Reclassifications

Certain reclassifications have been made to the prior year financial statements in order to conform to the current year's presentation.

### 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 May 2014, the Financial Accounting Standard Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-9, 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. On July 9, 2015, the FASB voted to defer the effective date by one year to December 15, 2017 for interim and annual reporting periods beginning after that date. Early adoption of ASU 2014-9 is permitted but not before the original effective date (annual periods beginning after December 15, 2016). We are currently evaluating the impact, if any, the adoption of this standard will have on our consolidated financial statements.

In July 2015, the FASB issued ASU No. 2015-11, Inventory (Topic 330): Simplifying the Measurement of Inventory. This standard amends Topic 330, Inventory, which currently requires an entity to measure inventory at the lower of cost or market. Market could be replacement cost, net realizable value, or net realizable value less an approximately normal profit margin. When this standard is adopted, an entity should measure in scope inventory at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The amendments are effective for fiscal years beginning after December 15, 2016, including interim periods within those fiscal years. We are currently evaluating the impact, if any, the adoption of this standard will have on our consolidated financial statements.

In November 2015, the FASB issued ASU No. 2015-17, Balance Sheet Classification of Deferred Taxes, to simplify the presentation of deferred taxes. This amendment requires that all deferred tax assets and liabilities, along with any related valuation allowances, be classified as noncurrent on the balance sheet. However, an entity shall not offset deferred tax liabilities and assets attributable to different tax jurisdictions. ASU 2015-17 is effective for annual and interim reporting periods ending after December 15, 2016. Early adoption is permitted, and the new guidance is and may be applied either prospectively or retrospectively. We have adopted this guidance prospectively as of December 31, 2015. Therefore, prior periods have not been adjusted to reflect this adoption. This change in accounting principle does not change our results of operations, cash flows or stockholders' equity.

In February 2016, the FASB issued Accounting Standards Update No. 2016-2, 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. We are currently evaluating the impact of our pending adoption of the new standard on our consolidated financial statements.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

Our primary exposure to market risk is related to changes in interest rates. As of December 31, 2015, we had cash equivalents and marketable securities of approximately \$199.0 million, consisting of money market funds, U.S. backed entity debt, corporate debt and commercial paper. This exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are

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in short-term marketable securities. Our marketable securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, a percent change in interest rates would have less than a \$2.0 million impact on our investments. We carry our investments based on publicly available information. We do not currently have any hard to value investment securities or securities for which a market is not readily available or active.

Item 8. Financial Statements and Supplementary Data

The consolidated financial statements and supplementary data of Retrophin, Inc. required by this Item are described in Item 15 of this Annual Report on Form 10-K and are presented beginning on page F-1.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure  
None.

Item 9A. Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our Exchange Act reports 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 year 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.

Management's Report on Internal Control Over Financial Reporting

Internal control over financial reporting refers to the process designed by, or under the supervision of, our Chief Executive Officer and Chief Financial Officer, and effected by our board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles, and includes those policies and procedures that:

- (1) Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;
- (2) Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorization of our management and directors; and
- (3) Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

Internal control over financial reporting cannot provide absolute assurance of achieving financial reporting objectives because of its inherent limitations. Internal control over financial reporting is a process that involves human diligence and compliance and is subject to lapses in judgment and breakdowns resulting from human failures. Internal control over financial reporting also can be circumvented by collusion or improper management override. Because of such limitations, there is a risk that material misstatements may not be prevented or detected on a timely basis by internal control over financial reporting. However, these inherent limitations are known features of the financial reporting process. Therefore, it is possible to design into the process safeguards to reduce, though not eliminate, this risk. Management is responsible for establishing and maintaining adequate internal control over financial reporting for the company.

Management has used the framework set forth in the report entitled Internal Control-Integrated Framework (2013 framework) published by the Committee of Sponsoring Organizations of the Treadway Commission, known as COSO, to evaluate the effectiveness of our internal control over financial reporting. Based on this assessment,

management has concluded that our internal control over financial reporting was effective as of December 31, 2015. BDO USA, LLP, our independent registered public accounting firm, has issued an attestation report on our internal control over financial reporting as of December 31, 2015, which is included herein.

**Remediation of Prior Year Material Weakness**

As disclosed in Item 9A. Controls and Procedures of Retrophin's Annual Report on Form 10-K as of December 31, 2014, management identified material weaknesses in our internal control over financial reporting. Throughout 2015, Retrophin's management designed and implemented a plan to remediate

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the deficiencies in the control environment. One such deficiency included a former member of senior management who did not demonstrate the appropriate level of control consciousness, reflect adequate tone at the top of the organization and who did not observe a diligent process relating to the review and approval of contracts. In addition, Retrophin's management also identified a material weakness in the control environment relating to the accounting for equity awards.

Through 2015, our management has taken the following actions that materially affect our internal control over financial reporting and remediate the material weaknesses described above.

• We have hired additional staff with expertise in applying complex accounting and financial reporting and disclosure rules required under U.S. GAAP and SEC reporting regulations, as well as allowing for segregation of duties.

• We appointed Gary A. Lyons, Timothy Coughlin, John Kozarich and Jeffrey Meckler as independent members of the Board of Directors.

• On November 10, 2014, Stephen Aselage became our Chief Executive Officer. Mr. Aselage has more than 30 years of pharmaceutical and biotechnology experience.

• On November 17, 2014, Laura Clague became our Chief Financial Officer. Mrs. Clague has extensive experience in accounting and finance, and pharmaceutical and biotechnology experience.

• On November 17, 2014, Margaret Valeur-Jensen, Ph.D. became our General Counsel. Ms. Valeur-Jensen has more than 25 years of experience working with public pharmaceutical and biotechnology companies.

On February 3, 2015, the Company held a Special Meeting of Stockholders at which the Company's stockholders voted to approve a proposal ratifying the prior issuance of stock options to purchase 1,928,000 shares of common stock and 230,000 restricted shares of common stock granted to employees between February 24, 2014 and August 18, 2014.

• In fiscal 2015, the Company instituted controls over the granting and tracking of stock options.

We achieved operational effectiveness with these controls and fully remediated the prior year material weaknesses during fiscal 2015.

**Changes In Internal Control Over Financial Reporting**

Other than as discussed above, there have not been any changes in our internal control over financial reporting during the quarter ended December 31, 2015 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.



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Report of Independent Registered Public Accounting Firm

Board of Directors and Stockholders

Retrophin, Inc.

San Diego, California

We have audited Retrophin, Inc. and its subsidiaries' (the "Company") internal control over financial reporting as of December 31, 2015, based on criteria established in Internal Control - Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (the COSO criteria). Retrophin Inc. and its subsidiaries' management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying, "Item 9A, Management's Report on Internal Control Over Financial Reporting." Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

In our opinion, Retrophin, Inc. and its subsidiaries maintained, in all material respects, effective internal control over financial reporting as of December 31, 2015, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of Retrophin, Inc. and its subsidiaries as of December 31, 2015 and 2014, and the related consolidated statements of operations and comprehensive income (loss), stockholders' equity (deficit), and cash flows for the years then ended and our report dated February 26, 2016 expressed an unqualified opinion thereon.

/s/ BDO USA LLP

New York, NY

February 26, 2016



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Item 9B. Other Information

None.

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PART III

Item 10. Directors, Executive Officers, and Corporate Governance

Information required by this item will be contained in our Definitive Proxy Statement for our 2016 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2015. Such information is incorporated herein by reference.

Item 11. Executive Compensation

Information required by this item will be contained in our Definitive Proxy Statement for our 2016 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2015. Such information is incorporated herein by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

Information required by this item will be contained in our Definitive Proxy Statement for our 2016 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2015. Such information is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence

Information required by this item will be contained in our Definitive Proxy Statement for our 2016 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2015. Such information is incorporated herein by reference.

Item 14. Principal Accounting Fees and Services

Information required by this item will be contained in our Definitive Proxy Statement for our 2016 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2015. Such information is incorporated herein by reference.

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PART IV

Item 15. Exhibits and Financial Statement Schedules

(a) (1) The financial statements at page F-1 are incorporated by reference to a part of this Annual Report on Form 10-K.

(2) Financial statement schedules are omitted because they are not applicable or the required information is shown in the financial statements or notes thereto.

(3) Exhibits: The exhibits to this report are listed in the exhibit index below.

(b) Description of Exhibits

Exhibit No. Description

2.1	Membership Interest Purchase Agreement, dated as of March 26, 2014, by and among Retrophin, Inc., on the one hand, and Loring Creek Holdings LLC, Lloyd Glenn and Matthias Kurth, on the other hand (incorporated by reference to Exhibit 10.2 to Company's Current Report on Form 8-K, filed with the Securities and Exchange Commission (the "SEC") on March 31, 2014).
2.2	Asset Purchase Agreement, dated May 22, 2015, by and between Retrophin, Inc. and Sanofi (incorporated by reference to Exhibit 2.1 to the Company's Current report on Form 8-K filed with the SEC on May 27, 2015).
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 Warrant issued to the purchasers in the private placement of 3,045,929 shares of common stock, dated February 14, 2013 (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on February 19, 2013).
4.3	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).
4.4	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).
4.5	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).
4.6	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 May 29, 2014).
4.7	Registration Rights Agreement, dated February 12, 2013, by and among the Company and the February 2013 Purchasers (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on February 19, 2013).
4.8	Registration Rights Agreement, dated August 15, 2013, by and among the Company and the August 2013 Purchasers (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on August 20, 2013).
4.90	

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- First Amendment to Registration Rights Agreement, dated August 14, 2013, by and among the Company and the purchasers signatory thereto (incorporated by reference to Exhibit 10.4 to the Company's Current Report on Form 8-K, filed with the SEC on August 20, 2013).
- 4.1 Form of Indenture for Senior Debt Securities (incorporated by reference to Exhibit 4.10 to the Company's Registration Statement on Form S-8, filed with the SEC on September 9, 2014).
- 4.11 Form of Indenture for Subordinated Debt Securities (incorporated by reference to Exhibit 4.11 to the Company's Registration Statement on Form S-8, filed with the SEC on September 9, 2014).
- 10.1 Separation Agreement and Release, dated September 15, 2014, by and between Retrophin, Inc. and Marc Panoff (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on September 16, 2014).
- 10.2 Form of Credit Agreement, dated as of June 30, 2014, among Retrophin, Inc., the lenders from time to time party thereto and U.S. Bank National Association, as Administrative Agent and Collateral Agent (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 13, 2014).
- 10.3 Form of Guarantee and Collateral Agreement, dated as of June 30, 2014, among Retrophin, Inc., the Guarantors from time to time party thereto and U.S. Bank National Association, as Collateral Agent (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on July 7, 2014).
- 10.4 First Amendment to Thiola® Trademark License and Supply Agreement, dated July 28, 2014 (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on July 29, 2014).

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10.5	Amendment No. 1 to Credit Agreement, dated July 16, 2014, among Retrophin, Inc., the lenders from time to time party thereto and U.S. Bank National Association, as Administrative Agent and Collateral Agent (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 13, 2014).
10.6	Amendment No. 2 to Credit Agreement, dated November 13, 2014, among Retrophin, Inc., the lenders from time to time party thereto and U.S. Bank National Association, as Administrative Agent and Collateral Agent (incorporated by reference to Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 13, 2014).
10.7	License Agreement, dated May 29, 2014, by and among Retrophin, Inc. and Mission Pharmacal Company (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).
10.8	First Amendment to Trademark License and Supply Agreement, effective as of July 28, 2014, by and between Mission Pharmacal Company and Retrophin, Inc. (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on July 29, 2014).
10.90	International Rights Purchase Agreement, dated as of March 26, 2014, by and between Manchester Pharmaceuticals LLC and Retrophin Therapeutics International, LLC (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on March 31, 2014).
10.10	Secured Promissory Note, dated March 26, 2014, made by Retrophin, Inc. in favor of Loring Creek Holdings LLC, Lloyd Glenn and Matthias Kurth (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K, filed with the SEC on March 31, 2014).
10.1	Membership Interest Pledge Agreement, dated as of March 26, 2014, by and between Retrophin, Inc., on the one hand, and Loring Creek Holdings LLC, Lloyd Glenn and Matthias Kurth, on the other hand (incorporated by reference to Exhibit 10.4 to the Company's Current Report on Form 8-K, filed with the SEC on March 31, 2014).
10.12	Security Agreement, dated as of March 26, 2014, by and between Manchester Pharmaceuticals LLC, on the one hand, and Loring Creek Holdings LLC, Lloyd Glenn and Matthias Kurth, on the other hand. (incorporated by reference to Exhibit 10.5 to the Company's Current Report on Form 8-K, filed with the SEC on March 31, 2014).
10.13+	Sublicense Agreement, dated February 16, 2012, by and among Ligand Pharmaceuticals Incorporated, a Delaware corporation, Pharmacopeia, Inc., a Delaware limited liability company, and Retrophin, LLC, a Delaware limited liability company (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on December 19, 2012).
10.14	Employment Agreement, dated April 24, 2013, by and between Retrophin, Inc. and Horacio Plotkin, M.D. (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on April 26, 2013).
10.15	Amendment to Employment Agreement, dated June 30, 2013, by and between Retrophin, Inc. and Marc Panoff (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on July 30, 2013).
10.16	Employment Agreement, dated March 2, 2015, by and between Retrophin, Inc. and Laura M. Clague (incorporated by reference to Exhibit 10.25 to the Company's Annual Report on Form 10-K, filed with the SEC on March 11, 2015).
10.17	Employment Agreement, dated March 2, 2015, by and between Retrophin, Inc. and Margaret Valeur-Jensen (incorporated by reference to Exhibit 10.26 to the Company's Annual Report on Form 10-K, filed with the SEC on March 11, 2015).
10.18	Employment Agreement, dated March 2, 2015, by and between Retrophin, Inc. and Stephen Aselage (incorporated by reference to Exhibit 10.27 to the Company's Annual Report on Form 10-K, filed with the SEC on March 11, 2015).
10.19	Summary Separation Proposal, dated October 13, 2014, by and between Retrophin, Inc. and Martin Shkreli (incorporated by reference to Exhibit 10.28 to the Company's Annual Report on Form 10-K,

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- filed with the SEC on March 11, 2015).
- 10.2 Retrophin, Inc. 2014 Incentive Compensation Plan as amended (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on February 9, 2015).
- 10.21 Retirement and Transition Agreement, dated February 1, 2016, by and between Retrophin, Inc. and Margaret Valeur-Jensen, Ph.D. (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on February 2, 2016).
- 10.22+ Amendment No. 4 to Sublicense Agreement dated as of September 17, 2015, between Retrophin, Inc. and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q/A, filed with the SEC on December 22, 2015).
- 10.23 Addendum to Trademark License and Supply Agreement, dated October 19, 2015, by and between to Company and Mission Pharmacal (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 6, 2015).
- 10.24 2015 Retrophin Inc. Executive/Designated 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 July 29, 2015).
- 10.25 Retrophin, Inc. 2015 Equity Incentive Plan, Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise, and Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement for use thereunder (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K filed with the SEC on June 11, 2015).
- 10.26 Asset Purchase Agreement dated as of January 9, 2015, between Retrophin, Inc. and Turing Pharmaceuticals AG (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
- 10.27 Asset Purchase Agreement dated as of February 12, 2015, among Retrophin, Inc., Manchester Pharmaceuticals LLC and Turing Pharmaceuticals AG (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
- 10.28 Asset Purchase Agreement dated as of February 12, 2015, between Retrophin, Inc. and Turing Pharmaceuticals AG (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
- 10.29 Amendment No. 3 to Credit Agreement dated January 12, 2015, among Retrophin, Inc., the lenders from time to time thereto and U.S. Bank National Association, as administrative agent and collateral agent (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).



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10.30+	Amendment No. 3 to Sublicense Agreement dated as of February 27, 2015, between Retrophin, Inc. and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.31+	Asset Purchase Agreement dated January 10, 2015 by and between Retrophin, Inc. and Asklepiion Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.6 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.32	Amendment No. 4 to Credit Agreement, dated March 24, 2015, among Retrophin, Inc., the lenders from time to time thereto and U.S. Bank National Association, as administrative agent and collateral agent (incorporated by reference to Exhibit 10.7 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.33	Employment Agreement, dated May 7, 2015, by and between Retrophin, Inc. and Alvin Shih (incorporated by reference to Exhibit 10.8 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.34	Purchase Agreement dated as of February 12, 2015 among Retrophin Inc., Manchester Pharmaceuticals LLC and Waldun Pharmaceuticals LLC (incorporated by reference to Exhibit 10.9 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.35	2016 Retrophin, Inc. Executive Officer Annual Bonus Plan (incorporated by reference to Exhibit 99.2 to the Company's Current Report on Form 8-K, filed with the SEC on February 25, 2016).
21.1	List of subsidiaries of the Company.
23.1	Consent of Marcum LLP.
23.2	Consent of BDO USA, LLP.
24.1	Power of Attorney (see signature page hereto).
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 Sarbanes Oxley Act of 2002.
32.2	Chief Financial Officer's Certification pursuant to Section 906 of 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.

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## SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: February 26, 2016

RETROPHIN, INC.

By: /s/ Stephen Aselage

Name: Stephen Aselage

Title: Chief Executive Officer

By: /s/ Laura Clague

Name: Laura Clague

Title: Chief Financial Officer

## POWER OF ATTORNEY

Know all men by these presents, that each person whose signature appears below constitutes and appoints Stephen Aselage and Laura Clague, and each of them, as his attorneys-in-fact and agents, each with power of substitution in any and all capacities, to sign any amendments to this annual report on Form 10-K, and to file the same with exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that the attorney-in-fact or his substitute or substitutes may do or cause to be done by virtue hereof. Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Stephen Aselage Stephen Aselage	Chief Executive Officer and Director (Principal Executive Officer)	February 26, 2016
/s/ Laura Clague Laura Clague	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	February 26, 2016
/s/ Timothy Coughlin Timothy Coughlin	Director	February 26, 2016
/s/ Cornelius Golding Cornelius Golding	Director	February 26, 2016
/s/ Jeffrey A. Meckler Jeffrey A. Meckler	Director	February 26, 2016
/s/ Gary Lyons Gary Lyons	Director	February 26, 2016
/s/ John Kozarich John Kozarich	Director	February 26, 2016



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RETROPHIN, INC. AND SUBSIDIARIES  
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Report of Independent Registered Public Accounting Firm

Board of Directors and Stockholders

Retrophin, Inc.

San Diego, CA

We have audited the accompanying consolidated balance sheets of Retrophin, Inc. and its subsidiaries (the “Company”) as of December 31, 2015 and 2014 and the related consolidated statements of operations and comprehensive income (loss), stockholders’ equity (deficit), and cash flows for the years then ended. These financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Retrophin, Inc. and its subsidiaries at December 31, 2015 and 2014, and the results of their operations and their cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), Retrophin Inc. and its subsidiaries’ internal control over financial reporting as of December 31, 2015, based on criteria established in Internal Control - Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) and our report dated February 26, 2016 expressed an unqualified opinion thereon.

/s/ BDO USA, LLP

New York, NY

February 26, 2016

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of  
Retrophin, Inc. and Subsidiary

We have audited the accompanying consolidated statements of operations and comprehensive loss, changes in stockholders' deficit and cash flows of Retrophin, Inc. and Subsidiary (the "Company") for the year ended December 31, 2013. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audit provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the consolidated results of its operations and its cash flows of Retrophin, Inc. and Subsidiary for the year ended December 31, 2013 in conformity with accounting principles generally accepted in the United States of America.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 3 to the December 31, 2013 consolidated financial statements (not presented herein), the Company is an early stage enterprise with no revenues, historical losses and limited capital resources. The Company, as an, early stage enterprise, is subject to risks and uncertainties as to whether it will be able to raise capital and commence its planned operations. These conditions raise substantial doubt about the Company's ability to continue as a going concern. Management's plans regarding these matters also are described in Note 3 to those consolidated financial statements. The consolidated financial statements do not include any adjustments that might be necessary should the Company be unable to continue as a going concern.

/s/ Marcum LLP

New York, NY

March 28, 2014, except for the information appearing in the first bullet point in Note 2 and the last five paragraphs of Note 13 to the consolidated financial statements (not presented herein) appearing under Item 8 of the Company 2013 Annual Report on Form 10-K/A Amendment No. 1 as to which the date is March 11, 2015.

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CONSOLIDATED BALANCE SHEETS

(In thousands, except share and per share amounts)

	December 31, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	\$37,805	\$18,204
Marketable securities	191,799	9,556
Accounts receivable, net	12,458	7,960
Inventory, net	2,536	801
Prepaid expenses and other current assets	2,378	813
Prepaid taxes	8,107	—
Note receivable, current	46,849	—
Total current assets	301,932	37,334
Property and equipment, net	428	671
Other asset	1,995	2,265
Intangible assets, net	161,536	94,265
Goodwill	936	936
Note receivable, long-term	45,573	—
Total assets	\$512,400	\$135,471
Liabilities and Stockholders' Deficit		
Current liabilities:		
Deferred technology purchase liability	\$—	\$1,000
Accounts payable	7,639	7,124
Accrued expenses	23,820	27,883
Guaranteed minimum royalty, short term	817	732
Other current liabilities	958	206
Business combination-related contingent consideration	13,754	2,118
Derivative financial instruments, warrants	38,810	27,990
Note payable	—	40,486
Total current liabilities	85,798	107,539
Convertible debt	43,902	43,288
Other noncurrent liabilities	3,066	1,617
Guaranteed minimum royalty, long term	10,068	10,617
Business combination-related contingent consideration, less current portion	45,267	9,520
Deferred income tax liability, net	24,328	141
Total liabilities	212,429	172,722
Stockholders' Equity (Deficit):		
Preferred stock Series A \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2015 and 2014, respectively	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 36,465,853 and 26,428,071 issued and 36,465,853 and 26,048,480 outstanding as of December 31, 2015 and 2014, respectively	4	3
Additional paid-in capital	365,802	140,851
Treasury stock, at cost, 0 and 379,591, respectively	—	(3,215 )
Accumulated deficit	(65,153	) (179,175 )
Accumulated other comprehensive income (loss)	(682	) 4,285

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Total stockholders' equity (deficit)	299,971	(37,251	)
Total liabilities and stockholders' equity (deficit)	\$512,400	\$135,471	

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

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

	Years Ended December 31,		
	2015	2014	2013
Net product sales	\$99,892	\$28,203	\$—
Operating expenses:			
Cost of goods sold	2,185	571	—
Research and development	50,426	47,795	7,084
Selling, general and administrative	79,541	59,645	17,689
Change in fair value of contingent consideration	13,778	—	—
Impairment of intangible assets	4,710	—	—
Total operating expenses	150,640	108,011	24,773
Operating loss	(50,748	) (79,808	) (24,773
Other expenses, net:			
Litigation settlement gain	15,500	—	—
Other income (expense), net	(296	) 2,352	370
Interest expense, net	(7,748	) (7,435	) (46
Debt early payment penalty	(2,250	) —	—
Loss on extinguishment of debt	(4,151	) —	—
Finance expense	(600	) (4,721	) —
Change in fair value of derivative instruments	(33,307	) (23,786	) (10,100
Gain on sale of assets	140,004	—	—
Bargain purchase gain	49,063	—	—
Total other income (expense), net	156,215	(33,590	) (9,776
Income (loss) before benefit (provision) for income taxes	105,467	(113,398	) (34,549
Income tax benefit (provision)	11,770	2,460	(76
Net income (loss)	\$117,237	\$(110,938	) \$(34,625
Net income (loss) per common share, basic	\$3.49	\$(4.43	) \$(2.44
Net income (loss) per common share, diluted	\$3.17	\$(4.43	) \$(2.44
Weighted average common shares outstanding, basic	33,560,249	25,057,509	14,205,264
Weighted average common shares outstanding, diluted	37,581,439	25,057,509	14,205,264
Comprehensive income (loss):			
Net income (loss)	\$117,237	\$(110,938	) \$(34,625
Foreign currency translation loss	(40	) —	—
Unrealized gain (loss) on sale of marketable securities	(4,927	) 4,396	(110
Comprehensive income (loss)	\$112,270	\$(106,542	) \$(34,735

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

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RETROPHIN, INC. AND SUBSIDIARIES  
CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' EQUITY (DEFICIT)  
FOR THE YEAR ENDED DECEMBER 31, 2015  
(In thousands, except share and per share amounts)

	Common Stock		Common Stock in Treasury		Additional paid in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total Stockholders' equity (deficit)
	Shares	Amount	Shares	Amount				
Balance - December 31, 2012	8,952,905	\$ 1	—	\$—	\$ 30,203	\$ —	\$ (33,612 )	\$ (3,408 )
Incentive shares granted-employees	135,000	—	—	—	—	—	—	—
Share based compensation-employees	—	—	—	—	1,424	—	—	1,424
Share based compensation-non employees	177,500	—	—	—	1,485	—	—	1,485
Consultants settlement	181,500	—	—	—	1,180	—	—	1,180
Incentive shares forfeited-employees	(20,833 )	—	—	—	—	—	—	—
Incentive shares forfeited-non employees	(37,500 )	—	—	—	—	—	—	—
Issuance of common stock in connection with January 2013 private placement at \$3.00 per share, net of fees of \$0	272,221	—	—	—	817	—	—	817
Issuance of common stock in connection with February 2013 private placement at \$3.00 per share, net of fees of \$0.9 million and registration payment obligation of \$0.4 million	3,045,929	—	—	—	2,441	—	—	2,441
Issuance of common stock in connection with August 2013 private placement at \$4.50 per share, net of fees of \$2.8 million and payment made to February investors for inducement to participate in August financing of \$2.2 million	5,531,401	1	—	—	10,670	—	—	10,671
Issuance of common stock in connection with payment made to February investors for inducement to participate in August financing, 271,222 shares at \$4.50 per share and	291,907	—	—	—	1,324	—	—	1,324

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20,685 shares at \$5.00 per share

Treasury stock	—	—	(130,790)	(957 )	—	—	—	(957 )
Shares issued on behalf of related party	11,000	—	—	—	81	—	—	81
Adjustment to existing shareholders	5,333	—	—	—	10	—	—	10
Unrealized loss on marketable securities	—	—	—	—	—	(110 )	—	(110 )
Net loss	—	—	—	—	—	—	(34,625 )	(34,625 )
Balance - December 31, 2013	18,546,363	2	(130,790)	(957 )	49,635	(110 )	(68,237 )	(19,667 )
Share based payments	730,774	—	—	—	16,639	—	—	16,639
Kyalin payments	96,628	—	—	—	1,000	—	—	1,000
Issuance of common stock in connection with January 2014 public offering at \$8.50 per share, net of fees of \$3.2 million	4,705,882	1	—	—	36,835	—	—	36,836
Exercise of warrants and reclassification of derivative liability	1,947,377	—	—	—	31,762	—	—	31,762
August 2013 private placement settlement	—	—	—	—	272	—	—	272
Treasury stock	—	—	(248,801)	(2,258)	—	—	—	(2,258 )
Issuance of common stock to convertible debt holders	401,047	—	—	—	4,708	—	—	4,708
Unrealized gain/(loss) on marketable securities	—	—	—	—	—	4,395	—	4,395
Net loss	—	—	—	—	—	—	(110,938 )	(110,938 )
Balance - December 31, 2014	26,428,071	3	(379,591)	(3,215)	140,851	4,285	(179,175 )	(37,251 )

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Share based payments	—	—	—	—	25,900	—	—	25,900
Vesting of stock for accrued severance	—	—	—	—	2,126	—	—	2,126
Issuance of common stock in connection with March 2015 public offering at \$19.00 per share, net of fees of \$9 million	7,866,000	1	—	—	139,986	—	—	139,987
Exercise of warrants and reclassification of derivative liability	870,306	—	—	—	28,012	—	—	28,012
Retirement of treasury stock	(379,591 )	—	379,591	3,215	—	—	(3,215 )	—
Unrealized gain/(loss) on marketable securities	—	—	—	—	—	(4,927 )	—	(4,927 )
Foreign currency translation adjustments	—	—	—	—	—	(40 )	—	(40 )
Option inducement liability reversal and adjustments	—	—	—	—	3,840	—	—	3,840
Issuance of common shares under the equity incentive plan	478,334	—	—	—	—	—	—	—
Shares issued in connection with Cholbam acquisition	661,279	—	—	—	15,844	—	—	15,844
Stock option exercises	541,454	—	—	—	6,818	—	—	6,818
Excess tax benefits of stock option exercises	—	—	—	—	2,425	—	—	2,425
Net Income	—	—	—	—	—	—	117,237	117,237
Balance - December 31, 2015	36,465,853	\$4	—	\$—	\$365,802	\$(682 )	\$(65,153)	\$299,971

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

Table of ContentsRETROPHIN, INC. AND SUBSIDIARIES  
CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	For the year ended December 31,		
	2015	2014	2013
<b>Cash Flows From Operating Activities:</b>			
Net income (loss)	\$ 117,237	\$(110,938 )	\$(34,625 )
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	13,392	5,401	216
Realized (gain) loss on marketable securities	293	(2,349 )	(374 )
Gain upon divestiture of Pediatric Priority Review Voucher	(140,004 )	—	—
Gain upon divestiture of assets to Turing Pharmaceuticals	(914 )	—	—
Deferred income tax	(15,573 )	(2,460 )	76
Settlement expense	—	5,746	—
Loss on extinguishment of debt	4,151	—	—
Impairment of intangible assets	4,710	—	—
Loss on disposal of fixed assets	112	—	—
Derivative financial instruments, warrants, issued, recorded in interest expense	1,050	—	—
Interest receivable from notes receivable	(1,267 )	—	—
Non-Cash interest expense	2,461	—	—
Amortization of debt discount and deferred financing costs	1,340	1,084	—
Amortization of premiums on investments	398	—	—
2013 private placement settlement	—	272	—
Non-cash financing cost	—	4,708	—
Loss on impairment of cost method purchase	—	400	—
Share based compensation	25,900	15,900	5,444
Shares issued on behalf of related party	—	—	81
Registration payment obligation expense	—	—	360
Reversal of registration payment obligation liability	—	—	(360 )
Bargain purchase gain	(49,063 )	—	—
Change in estimated fair value of contingent consideration, net of payments	13,288	—	—
Change in estimated fair value of liability classified warrants	33,307	23,786	10,100
Changes in operating assets and liabilities, net of business acquisitions:			
Accounts receivable	(4,504 )	(7,959 )	—
Inventory	(1,174 )	(282 )	—
Prepaid expenses and other current assets	(966 )	237	(1,349 )
Prepaid income taxes	(8,107 )	—	—
Accounts payable and accrued expenses	3,379	20,604	2,842
Net cash used in operating activities	(554 )	(45,850 )	(17,589 )
<b>Cash Flows From Investing Activities:</b>			
Purchase of fixed assets	(22 )	(663 )	(117 )
Purchase of intangible assets	(7,008 )	(3,301 )	(5,433 )
Security deposits	—	(93 )	(106 )
Repayment of technology license liability	—	—	(1,300 )
Proceeds from the sale/maturity of marketable securities	9,977	6,493	4,385
Purchase of marketable securities	(198,530 )	(10,149 )	(4,124 )

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Proceeds from securities sold, not yet purchased	—	7,500	4,194	
Securities sold, not yet purchased	—	(7,500	) (2,865	)
Increase in restricted cash	—	—	(40	)
Cash received upon sale of assets, net	148,411	—	—	
Cash paid for investment	—	(400	) —	
Cash paid upon acquisition, net of cash acquired	(33,430	) (29,150	) —	
Net cash used in investing activities	(80,602	) (37,263	) (5,406	)
Cash Flows From Financing Activities:				
Repayment of net amounts due to related parties	—	—	(13	)
Payment of acquisition-related contingent consideration	(3,938	) (1,163	) —	
Payment of other liability	(2,000	) (500	) —	
Payment of guaranteed minimum royalty	(2,000	) —	—	
Repayment of note payable - related party	—	—	(885	)
Investors' deposits	—	—	(100	)

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Proceeds from credit agreement	—	42,366	—
Proceeds from Note Purchase Agreement	—	42,924	—
Proceeds from exercise of warrants	4,475	8,398	—
Proceeds from exercise of stock options	6,818	—	—
Repayment of Manchester note payable	—	(31,283	) —
Excess tax benefit related to stock compensation	2,425	—	—
Proceeds received from issuance of common stock	149,487	40,000	30,937
Financing costs from issuance of common stock	(9,500	) (3,165	) —
Repayment of credit facility	(45,000	) —	—
Purchase of treasury stock, at cost	—	(2,257	) (958
Net cash provided by financing activities	100,767	95,320	28,981
Effect of exchange rate changes on cash	(10	) —	—
Net increase in cash and cash equivalents	19,601	12,207	5,986
Cash and cash equivalents, beginning of year	18,204	5,997	11
Cash and cash equivalents, end of year	\$37,805	\$18,204	\$5,997
Supplemental Disclosure of Cash Flow Information:			
Cash paid for interest	\$5,838	\$4,080	\$28
Cash paid for income taxes	\$9,610	\$5	\$—
Non-cash Investing and financing activities:			
Accrued royalty in excess of minimum payable to the sellers of Thiola	\$8,219	\$—	\$—
Present value of contingent consideration payable to sellers of Asklepiion Pharmaceuticals LLC	\$42,010	\$—	\$—
Shares issued in connection with Cholbam acquisition	\$15,844	\$—	\$—
Reclassification of derivative liability to equity due to exercise of warrants	\$23,537	\$23,365	\$—
Present value of contingent consideration payable to sellers of Manchester Pharmaceuticals, LLC.	\$—	\$12,800	\$—
Present value of guaranteed minimum royalty payable to sellers of Thiola	\$—	\$11,850	\$—
Note payable entered into upon consummation of Manchester Pharmaceuticals, LLC.	\$—	\$31,283	\$—
Unrealized loss on securities sold, not yet purchased	\$—	\$—	\$(113
Adjustment to existing shareholders	\$—	\$—	\$10
Purchase of Kyalin in exchange for future consideration	\$—	\$1,000	\$2,635
Affiliate receivable applied to security deposit	\$—	\$—	\$138
Share based payment made to investors for inducement to participate in financing	\$—	\$—	\$1,324
Offering expense liability	\$—	\$—	\$747
Increase in basis of indefinite lived intangible assets acquired from Kyalin due to accrual of deferred tax liability	\$—	\$—	\$2,525

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



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RETROPHIN, INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1. DESCRIPTION OF BUSINESS

Organization and Description of Business

Retrophin, Inc. (“we”, “our”, “us”, “Retrophin” and the “Company”) refers to Retrophin, Inc., a Delaware corporation, as well as our direct and indirect subsidiaries. Retrophin is a fully integrated biopharmaceutical company headquartered in San Diego, California focused on the development, acquisition and commercialization of therapies for the treatment of serious, catastrophic or 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 serious, catastrophic or rare diseases and that we believe offer attractive growth characteristics.

During the first quarter of 2015, the Company completed the acquisition of all worldwide rights, titles, and ownership of Cholbam (cholic acid), the first FDA approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for patients with peroxisomal disorders (including Zellweger spectrum disorders). The Company generated the first sales from Cholbam (known as Kolbam in the European Union) in April 2015. See Note 3. for further discussion.

We currently sell the following three products:

Chenodal® (chenodeoxycholic acid) is approved in the United States for the treatment of patients suffering from gallstones in whom surgery poses an unacceptable health risk due to disease or advanced age. Chenodal has been the standard of care for cerebrotendinous xanthomatosis (“CTX”) patients for more than three decades and the Company is currently pursuing adding this indication to the label.

Cholbam (cholic acid) is approved in the United States for the treatment of bile acid synthesis disorders due to single enzyme defects and is further indicated for adjunctive treatment of patients with peroxisomal disorders.

Thiola (tiopronin) is approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.

We are currently developing the following pipeline products:

Sparsentan, also known as RE-021, is an investigational therapeutic agent which acts as both a potent angiotensin receptor blocker (“ARB”), as well as a selective endothelin receptor antagonist (“ERA”), with selectivity toward endothelin receptor type A. We have secured a license to sparsentan from Ligand and Bristol-Myers Squibb (who referred to it as DARA). We are developing sparsentan as a treatment for FSGS, which is a leading cause of end-stage renal disease. We are currently enrolling patients for the DUET Phase 2 clinical study of sparsentan for the treatment of FSGS and we anticipate having a top line data read out in the third quarter of 2016. Depending on the data obtained in the DUET study, we may be able to support an application for accelerated approval for sparsentan on the basis of proteinuria as a surrogate endpoint. Sparsentan was granted fast track designation in June 2015 and orphan drug designation in the U.S. and EU in January and November 2015, respectively.

RE-024, a novel small molecule, is being developed 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 parkinsonism, dystonia, and other severe systemic manifestations. There are currently no viable treatment options for patients with PKAN. RE-024 is a phosphopantothenate prodrug therapy that aims to restore levels of this key substrate in PKAN patients. Certain international health regulators have approved the initiation of dosing RE-024 in PKAN under physician-initiated studies in accordance with local regulations in their respective countries. The Company filed a U.S. IND for RE-024 with the FDA in the first quarter of 2015 to support the commencement of a Company-sponsored Phase 1 study, which initiated in April 2015. RE-024 was granted orphan drug designation from the FDA in May 2015 and was granted fast track designation in June 2015. RE-034 is a synthetic hormone analog of the first 24 amino acids of the 39 amino acids contained in adrenocorticotrophic hormone (“ACTH”) incorporated into a novel formulation developed by the Company. RE-034 exhibits similar physiological actions as endogenous ACTH by binding to melanocortin receptors, resulting in its anti-inflammatory and immunomodulatory effects. The Company has successfully manufactured RE-034 at proof-of-concept scale using a novel formulation that allows modulation of the release of the active ingredient from the site of administration. The Company intends to continue preclinical development of RE-034 to enable multiple

strategic options.

**2015 Public Offering**

On March 24, 2015, we completed a public offering of 7,866,000 shares of common stock at a price of \$19.00 per share. We received net proceeds from the offering of \$140.0 million, after deducting underwriting fees and other offering costs of \$9.5 million. The shares of common stock were offered by us pursuant to a shelf registration statement that was declared effective by the SEC on March 13, 2015.

**NOTE 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES**

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

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### Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with accounting principles generally accepted in the United States ("U.S. GAAP"). All intercompany accounts and transactions have been eliminated in consolidation.

### Use of Estimates

In preparing financial statements in conformity with U.S. GAAP, management is required to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting period. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions. These estimates and assumptions include revenue recognition, valuing equity securities in share-based payments, estimating fair value of equity instruments recorded as derivative liabilities, estimating the fair value of net assets acquired in business combinations, estimating the useful lives of depreciable and amortizable assets, goodwill impairment, estimating of contingent consideration, estimating of valuation allowances and uncertain tax positions, and estimating the fair value of long-lived assets to assess whether impairment charges may apply.

### Revenue Recognition

Product sales for the year ended December 31, 2015 consisted of sales of Chenodal, Vecamyl (divested in 2015), Cholbam and Thiola. Product sales for the year ended December 31, 2014 consisted of sales of Chenodal, Vecamyl and Thiola. Revenue from product sales is recognized when persuasive evidence of an arrangement exists, title to product and associated risk of loss have passed to the customer, the price is fixed or determinable, collection from the customer is reasonably assured, the Company has no further performance obligations, and returns can be reasonably estimated. The Company sells in the United States and Canada through a direct-to-patient distributor. Under this distribution model, the Company records revenues when the distributor ships products to customers and such customers take title of the product.

The Company sells internationally, but these revenues are immaterial.

Revenue from product sales is recorded net of applicable provisions for rebates under government programs (including Medicaid), prompt pay discounts, and other sales-related deductions. We review our estimates of rebates and other applicable provisions each period and record any necessary adjustments in the current period.

### Deductions from Revenue

**Government Rebates and Chargebacks:** The Company estimates the rebates that we will be obligated to provide to government programs and deducts 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.

**Prompt Pay Discounts:** The Company offers discounts to certain customers for prompt payments. The Company estimates these discounts based on customer terms and historical trends. The Company accrues for the estimated 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, the Company offers its 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 so returns are immaterial.

### Research and Development Costs

Research and development costs are expensed as incurred and include: salaries, benefits, bonus, stock-based compensation, license fees, milestone payments due under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials and delivery devices, and associated overhead and facilities costs. Clinical trial costs are a significant component of research and development expenses and include costs associated with third-party contractors, and clinical research organizations ("CRO's"). Invoicing from third-party contractors for services performed can lag several months. We accrue the costs of services rendered in connection with third-party contractor activities based on our estimate of management fees, and costs associated with site monitoring and data management.

### Employee Stock-Based Compensation

The Company recognizes all employee share-based compensation as a cost in the financial statements. Equity-classified awards principally related to stock options, restricted stock units ("RSUs") and performance stock units ("PSUs"), are measured at the grant date fair value of the award. The Company determines grant date fair value of stock option awards using the Black-Scholes option-pricing model. The fair value of RSUs are determined using the closing price of the Company's common stock on the grant date. For service based vesting grants, expense is recognized over the requisite service period based on the number of options or shares expected to ultimately vest. For PSUs, expense is recognized over the implicit period until the performance obligation is met, assuming that it is probable. No expense is recognized for PSUs until it is probable the vesting criteria will be satisfied. Forfeitures are estimated at the date of grant and revised when actual or expected forfeiture activity differs materially from original estimates.

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Stock Options	Vesting Term 1 to 3 years
Restricted Stock Units	1 to 3 years
Earnings (Loss) Per Share	
We calculate our basic earnings per share by dividing net income by the weighted average number of shares outstanding during the period. The diluted earnings per share computation includes the effect, if any, of shares that would be issuable upon the exercise of outstanding stock options, derivative liability, convertible debt and RSUs, reduced by the number of shares which are assumed to be purchased by the Company from the resulting proceeds at the average market price during the year, when such amounts are dilutive to the earnings per share calculation.	
Cash and Cash Equivalents	
We consider all highly liquid marketable securities with an original maturity of three months or less to be cash equivalents. Due to the short-term maturity of such investments, the carrying amounts are a reasonable estimate of fair value.	
Marketable Securities	
The Company accounts for marketable securities held as “available-for-sale” in accordance with ASC 320, “Investments Debt and Equity Securities” (“ASC 320”). The Company classifies these investments as current assets and carries them at fair value. Unrealized gains and losses are recorded as a separate component of stockholders’ equity as accumulated other comprehensive income (loss). Realized gains or losses on marketable security transactions are reported in the Statements of Operations and Comprehensive Income (Loss). Marketable securities are maintained at one financial institution and are governed by the Company’s investment policy as approved by our Board of Directors. Fair values of marketable securities are based on quoted market prices.	
Trade and Notes Receivable	
Trade Receivables, Net	
Trade accounts receivable are recorded net of allowances for prompt payment and doubtful accounts. Allowances for rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets. Estimates for allowances for doubtful accounts are determined based on existing contractual obligations, historical payment patterns and individual customer circumstances. The allowance for doubtful accounts was \$0 and \$0.1 million at December 31, 2015 and 2014, respectively. The bad debt expense recorded in the Statement of Operations and Comprehensive Income (Loss) is approximately \$0 million and \$0.1 million for 2015 and 2014, respectively.	
Notes Receivable	
Notes receivable arose from the sale of the pediatric priority review voucher (the "PRV"). On July 2, 2015, the Company sold and transferred the PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million is due on each of the first and second anniversaries of the closing. In accordance with U.S. GAAP, the Company recorded the future short term and long term notes receivable at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale using a discount rate of 2.8%. The accretion on the notes receivables totaled \$1.3 million and is recorded in interest expense, net, in the Consolidated Statements of Operations and Comprehensive Income (Loss) for 2015. As of December 31, 2015, the present value of the current and long-term notes receivable was \$46.8 million and \$45.6 million, respectively. The Company noted no indications for impairment as of December 31, 2015.	
Inventories and Related Reserves	
Inventory is stated at the lower of cost or market. The Company determines the cost of inventory using the first-in, first-out, or FIFO, method. The Company periodically analyzes its inventory levels to identify inventory that may expire prior to expected sale or has a cost basis in excess of its estimated realizable value, and writes down such inventory as appropriate. In addition, the Company’s products are subject to strict quality control and monitoring which the Company’s manufacturers perform throughout their manufacturing process. The Company does not directly manufacture any product. The Company has single suppliers for products Chenodal and Thiola, and prospectively arranges for manufacture from contract service providers for its product Cholbam. The value of inventory acquired in 2015 related to single supplier purchases was 63% for Thiola and 4% for Chenodal. The remaining 33% of inventory was related to the Cholbam product and was either related to materials acquired or subsequent third party	

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manufacturing. The inventory reserve was \$0.3 million and \$0.1 at December 31, 2015 and 2014, respectively. Inventory, net of reserve, consists of the following at December 31, 2015 and 2014 (in thousands):

	December 31, 2015	December 31, 2014
Raw material	\$289	\$315
Finished goods	2,247	486
Total inventory	\$2,536	\$801
Property and Equipment, net		

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Property, plant and equipment are stated at cost, net of accumulated depreciation. Depreciation is computed using the straight-line method over the related estimated useful lives as presented in the table below. Significant additions and improvements are capitalized, while repairs and maintenance are charged to expense as incurred. Property and equipment purchased for specific research and development projects with no alternative uses is expensed as incurred. The major classifications of property and equipment, including their respective expected useful lives, consists of the following:

Computer equipment	3 years
Furniture and fixtures	7 years
Leasehold improvements	Shorter of length of lease or life of the asset

**Intangible Assets, Net**

Intangible assets with finite useful lives consist primarily of product rights, licenses and customer relationships which are amortized on a straight line basis over 1 to 16 years. Intangible assets with finite useful lives are reviewed for impairment in accordance with ASC 360 and the useful lives are reassessed whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. If circumstances require a long-lived asset or asset group be tested for possible impairment, the Company first compares undiscounted cash flows expected to be generated by that asset or asset group to its carrying value. If the carrying value of the long-lived asset or asset group is not recoverable on an undiscounted cash flow basis, an impairment is recognized to the extent that the carrying value exceeds its fair value. The Company reviews for indications of impairment of intangibles on a quarterly basis.

For the year ended December 31, 2015 the company wrote off the intangible asset related to Carbetocin and recorded a loss of \$4.7 million. There were no impairments related to intangible assets for 2014 or 2013.

**Goodwill**

Goodwill represents the future economic benefits arising from assets acquired in a business combination that are not individually identified and separately recognized. The Company first assesses the qualitative factors for reporting units that carry goodwill. If the qualitative assessment results in a conclusion that it is more likely than not that the fair value of a reporting unit exceeds the carrying value, then no further testing is performed for that reporting unit. When a qualitative assessment is not used, or if the qualitative assessment is not conclusive and it is necessary to calculate fair value of a reporting unit, then the impairment analysis for goodwill is performed at the reporting unit level using a two-step approach. The first step of the goodwill impairment test is used to identify potential impairment by comparing the fair value of a reporting unit with its carrying amount, including goodwill utilizing an enterprise-value based premise approach. If the fair value of the reporting unit exceeds its carrying value, step two does not need to be performed. If the fair value of the reporting unit is less than its carrying value, an indication of goodwill impairment exists for the reporting unit and the entity must perform step two of the impairment test (measurement). Under step two, an impairment loss is recognized for any excess of the carrying amount of the reporting unit's goodwill over the implied fair value of that goodwill. The implied fair value of goodwill is determined by allocating the fair value of the reporting unit in a manner similar to a purchase price allocation and the residual fair value after this allocation is the implied fair value of the reporting unit goodwill. Fair value of the reporting unit is determined by using various valuation techniques including income (discounted cash flow), market and/or consideration of recent and similar purchase acquisition transactions. The Company performs its annual impairment review of goodwill in the fourth quarter and when a triggering event occurs between annual impairment tests. The Company has one segment and one reporting unit and as such reviews goodwill as one unit.

For the years ended December 31, 2015 and 2014 there were no impairments to goodwill.

**Income Taxes**

The Company follows ASC 740, Income Taxes, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are based on the differences between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance to the extent management concludes it is more likely

than not that the asset will not be realized.

The standard addresses the determination of whether tax benefits claimed or expected to be claimed on a tax return should be recorded in the financial statements. Under ASC 740, the Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the tax authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position should be measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement. ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. The Company's policy is to record estimated interest and penalty related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision.

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### Reclassifications

Certain reclassifications have been made to the prior year financial statements in order to conform to the current year's presentation.

### Patents

The Company expenses external costs, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company also expenses costs associated with maintaining and defending patents subsequent to their issuance in the period incurred.

### Derivative Instruments

The Company does not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. The Company evaluates all of its financial instruments to determine if such instruments are derivatives or contain features that qualify as embedded derivatives. For derivative financial instruments that are accounted for as liabilities, the derivative instrument is initially recorded at its fair value and is then revalued at each reporting date, with changes in the fair value reported in the statements of operations. For stock-based derivative financial instruments, the Company calculates the fair value of the financial instruments using the Monte Carlo simulation pricing model, however, prior to January 1, 2015, the Company used the Binomial Lattice option pricing model. The classification of derivative instruments, including whether such instruments should be recorded as liabilities or as equity is assessed at inception, the fair value of the warrants is evaluated at the end of each reporting period (see Note 5 and Note 6).

### Treasury Stock

The Company records treasury stock at the cost to acquire it and includes treasury stock as a component of stockholders' equity until it is retired.

### 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 May 2014, the Financial Accounting Standard Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-9, 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. On July 9, 2015, the FASB voted to defer the effective date by one year to December 15, 2017 for interim and annual reporting periods beginning after that date. Early adoption of ASU 2014-9 is permitted but not before the original effective date (annual periods beginning after December 15, 2016). We are currently evaluating the impact, if any, the adoption of this standard will have on our consolidated financial statements.

In July 2015, the FASB issued ASU No. 2015-11, Inventory (Topic 330): Simplifying the Measurement of Inventory. This standard amends Topic 330, Inventory, which currently requires an entity to measure inventory at the lower of cost or market. Market could be replacement cost, net realizable value, or net realizable value less an approximately normal profit margin. When this standard is adopted, an entity should measure in scope inventory at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The amendments are effective for fiscal years beginning after December 15, 2016, including interim periods within those fiscal years. We are currently evaluating the impact, if any, the adoption of this standard will have on our consolidated financial statements.

In November 2015, the FASB issued ASU No. 2015-17, Balance Sheet Classification of Deferred Taxes, to simplify the presentation of deferred taxes. This amendment requires that all deferred tax assets and liabilities, along with any related valuation allowances, be classified as noncurrent on the balance sheet. However, an entity shall not offset deferred tax liabilities and assets attributable to different tax jurisdictions. ASU 2015-17 is effective for annual and interim reporting periods ending after December 15, 2016. Early adoption is permitted, and the new guidance may be applied either prospectively or retrospectively. We have adopted this guidance prospectively as of December 31, 2015. Therefore, prior periods have not been adjusted to reflect this adoption. This change in accounting principle does not change our results of operations, cash flows or stockholders' equity.

In February 2016, the FASB issued Accounting Standards Update 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. We are currently evaluating the impact of our pending adoption of the new standard on our consolidated financial statements.

**NOTE 3. BUSINESS COMBINATION AND DIVESTITURE OF ASSETS**

**Acquisition of Cholic Acid**

On January 12, 2015, the Company announced the signing of a definitive agreement under which it acquired the exclusive right to purchase from Asklepion, all worldwide rights, titles, and ownership of Cholbam (cholic acid) for the treatment of bile acid synthesis defects, if approved by the FDA.

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Under the terms of the agreement, Retrophin paid Asklepiion an upfront payment of \$5.0 million and agreed to pay milestones based on FDA approval and net product sales, plus tiered royalties on future net sales of Cholbam. On March 18, 2015, the Company announced that the FDA had approved Cholbam capsules, the first FDA approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for patients with peroxisomal disorders (including Zellweger spectrum disorders). As a result of the approval, Retrophin exercised its right to purchase from Asklepiion all worldwide rights, titles, and ownership of Cholbam and related assets. The FDA also granted Asklepiion a Pediatric PRV, awarded to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A Pediatric PRV is transferable and provides the bearer with FDA priority review classification for a new drug application. The Pediatric PRV was transferred to Retrophin under the original terms of the agreement with Asklepiion.

On March 31, 2015, the Company completed its acquisition from Asklepiion of all worldwide rights, titles and ownership of Cholbam, including all related contracts, data assets, intellectual property, regulatory assets and the Pediatric PRV, in exchange for a cash payment of \$28.4 million, in addition to approximately 661,279 shares of the Company's common stock (initially valued at \$9 million at the time of the definitive agreement with Asklepiion, and \$15.8 million at the acquisition completion date). The Company is also required to pay contingent consideration consisting of milestones and tiered royalties with a present value of \$39.1 million.

The original asset value of the Pediatric PRV was recognized at \$96.3 million. In this valuation process, we considered various factors which included data from recent sales of similar vouchers. The consideration paid to Asklepiion did not value the Pediatric PRV because the issuance of a Pediatric PRV is extremely rare. Therefore when the FDA granted the Pediatric PRV with the Cholbam approval, a bargain purchase gain resulted.

The acquisition was accounted for under the purchase method of accounting in accordance with ASC 805. The fair value of assets acquired and liabilities assumed was based upon valuation and the Company's estimates. Critical estimates in valuing certain intangible assets include but are not limited to future expected cash flows from acquired product rights-Cholbam, Pediatric PRV, trade names and developed technologies, present value and discount rates. Management's estimates of fair value are based upon assumptions believed to be reasonable, but which are inherently uncertain and unpredictable and, as a result, actual results may differ from estimates.

The purchase included \$83.2 million of intangible assets with definite lives related to product rights with values of \$75.9 million for the U.S. and \$7.3 million for the international rights. The useful lives related to the acquired product rights are expected to be approximately 10 years.

The contingent consideration of \$39.1 million recorded during the year ended December 31, 2015 is related to an agreement to pay an additional cash amount based on the product performance through 2025. The accrued contingent consideration was recorded as a liability at acquisition-date fair value using the income approach with assumed discount rates of 19.0% over the applicable term. The undiscounted amount the Company could pay under the contingent consideration agreement is up to \$16.3 million.

Service fees with a net present value of \$2.9 million were recorded during the year ended December 31, 2015. The net present value is based upon \$4.0 million in total payments over a four years period starting as of the acquisition date. As part of the business combination the Company recorded a deferred tax liability of \$39.9 million. The deferred tax liability is derived from the difference in the Company's book basis and tax basis in the assets acquired of \$88.5 million. Our tax rate utilized is 45.4%. This reduced the Company's deferred tax asset, see Note 14.

The purchase price allocation of \$91.3 million as of the acquisition completion date of March 31, 2015 is as follows (in thousands):

Cash paid upon consummation	\$33,430
Present value of contingent consideration and service fees	42,010
Fair Value of 661,279 shares issued to Asklepiion	15,844
Total Purchase Price	\$91,284

Fair Value of Assets Acquired and Liabilities Assumed

Acquired product rights-Cholbam (Intangible Asset)	\$83,200
Pediatric Priority Review Voucher	96,250

Inventory	777	
Deferred tax liability	(39,880	)
Total Allocation of Purchase Price	\$140,347	
Bargain Purchase Gain	(49,063	)
Total Purchase Price	\$91,284	

Unaudited pro forma information for the transaction is not presented, because the effects of such transaction is considered immaterial to the Company.

#### Acquisition of Manchester Pharmaceuticals LLC

On March 26, 2014 (the “Manchester Closing Date”), the Company acquired 100% of the outstanding membership interests of Manchester. Under the terms of the agreement, the Company paid \$29.2 million upon consummation of the transaction, of which \$3.2 million was paid by Retrophin Therapeutics International LLC, an indirect wholly owned subsidiary, for rights of product sales outside of the United States. Acquisition costs amounted to approximately \$0.3 million and were recorded as selling, general, and administrative expense in the 2014 consolidated financial statements. The Company entered into a promissory note with Manchester for \$33 million which was discounted to \$31.3 million to be paid in three equal installments of \$11 million within

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three, six, and nine months after the Manchester Closing Date. On June 30, 2014, the Company paid the sellers of Manchester \$33 million in full satisfaction of the outstanding amount owed.

In addition, the Company agreed to make contractual payments based on 10% of net sales of the products Chenodal and Vecamyl to the former members of Manchester. Additional contingent payments will be made based on 5% of net sales from any new products derived from Chenodal and Vecamyl. Business combination-related contingent consideration estimated at \$12.8 million will be revalued at each reporting period and any change in valuation will be recorded in the Company's statement of operations.

The acquisition was accounted for under the purchase method of accounting in accordance with ASC 805, with the excess purchase price over the fair market value of the assets acquired and liabilities assumed allocated to goodwill. Based on the purchase price allocation, the purchase price of \$73.2 million resulted in goodwill of \$0.9 million which is primarily attributed to the synergies expected to arise after the acquisition. The \$0.9 million of goodwill resulting from the acquisition is deductible for income tax purposes.

Critical estimates in valuing certain intangible assets include but are not limited to future expected cash flows from customer relationships and developed technology, present value and discount rates. Management's estimates of fair value are based upon assumptions believed to be reasonable, but which are inherently uncertain and unpredictable and, as a result, actual results may differ from estimates.

The purchase included \$72 million of intangible assets with definite lives related to product rights, trade names, and customer relationships with values of \$71.4 million, \$0.2 million, and \$0.4 million, respectively. The useful lives related to the acquired product rights, trade names, and customer relationships are approximately 16, 1 and 10 years, respectively. Under the terms of the agreement, the sellers agreed to indemnify the Company for uncertain tax liabilities, any breach of any representation or warranty the sellers made to the purchaser, failure of the sellers to perform any covenants or obligations made to the purchaser, and third party claims relating to the operation of the Company and events occurring prior to the Manchester Closing Date. As of December 31, 2014, the Company recorded an indemnification asset with a corresponding liability in the amount of \$1.5 million related to uncertain tax liabilities.

The purchase price allocation of \$73.2 million as of the Manchester Closing Date was as follows:

	Amount (in thousands)
Cash paid upon consummation, net	\$29,150
Secured promissory note	31,283
Fair value of business combination-related contingent consideration	12,800
Total purchase price	\$73,233
Prepaid expenses	\$116
Inventory	517
Product rights	71,372
Trade names	175
Customer relationship	403
Goodwill	936
Other asset	1,522
Accounts payable and accrued expenses	(286)
Other liability	(1,522)
Total allocation of purchase price consideration	\$73,233

Divestiture of Assets:

Sale of Assets to Sanofi

The FDA granted Asklepiion Pharmaceuticals, LLC a Rare Pediatric Disease Priority Review Voucher ("Pediatric PRV"), awarded to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A Pediatric PRV is transferable and provides the bearer with FDA priority review classification for a new drug application. The Pediatric PRV was transferred to Retrophin under the terms of the asset purchase agreement between the Company and Asklepiion dated January 12, 2015, pursuant to which the Company acquired

Cholbam.

On July 2, 2015, the Company sold and transferred the Pediatric PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million is due on each of the first and second anniversaries of the closing. In accordance with U.S. GAAP, the Company recorded the future short term and long term notes receivable at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale using a discount rate of 2.8%. The gain from the sale of the asset was approximately \$140.0 million, net of \$4.9 million in fees contractually due as part of the Cholbam acquisition.

Sale of Assets to Turing Pharmaceuticals

On October 13, 2014, the Company entered into a binding Summary Separation Proposal with its then-current Chief Executive Officer. Among other things, the Summary Separation Proposal set forth a summary of the terms for the sale of the Company's Vecamyl, Syntocinon and ketamine licenses and assets to Turing Pharmaceuticals, a company controlled by the former Chief Executive Officer.

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On January 9, 2015, the Company entered into a purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals the Sold Assets for a purchase price of \$1.0 million, and pursuant to which Turing Pharmaceuticals also assumed all future liabilities related to the Sold Assets.

On February 13, 2015, the Sellers entered into a purchase agreement with Waldun, pursuant to which the Sellers sold Waldun the Vecamyl Product Rights for a purchase price of \$0.7 million. Waldun in turn sold the Vecamyl Product Rights to Turing Pharmaceuticals. In connection therewith, on February 13, 2015, the Company, together with Manchester, entered into an asset purchase agreement with Turing Pharmaceuticals, pursuant to which the Company sold Turing Pharmaceuticals the Inventory for a purchase price of \$0.3 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Vecamyl Product Rights and the Inventory.

On February 13, 2015, the Company entered into an asset purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals its Oxytocin Assets, including related inventory, for a purchase price of \$1.1 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Oxytocin Assets. See Note 16 for further discussion.

The effect on the Statement of Operations and Comprehensive Income (Loss) for 2015 is a gain of approximately \$0.9 million. See Note 9. to the financial statements for more information.

**NOTE 4. MARKETABLE SECURITIES**

The Company's marketable securities as of December 31, 2015 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 (loss). The amortized cost of debt securities in this category are adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion is included in interest 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. The cost of securities sold is based on the specific identification method. Interest and dividends on securities classified as available-for-sale are included in interest income. As of December 31, 2014, the Company owned available-for-sale marketable equity securities that were carried at fair value which have subsequently been sold.

Marketable securities consist of the following (in thousands):

	December 31, 2015	2014
Marketable Equity Securities:		
Common Stock	\$—	\$9,556
Marketable Other than Equity Securities:		
Commercial paper	31,864	—
Corporate debt securities	125,547	—
Securities of government sponsored entities	34,388	—
Total Marketable Securities:	\$191,799	\$9,556

The following is a summary of short-term marketable securities classified as available-for-sale as of December 31, 2015 (in thousands):

	Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable Other than Equity Securities:					
Commercial paper	Less than 1	\$31,899	\$6	\$(41)	\$31,864
Corporate debt securities	Less than 1	43,464	—	(78)	43,386
Total maturity less than 1 year		75,363	6	(119)	75,250
Corporate debt securities	1 to 2	82,557	—	(396)	82,161
Securities of government-sponsored entities	1 to 2	34,522	2	(136)	34,388
Total maturity 1 to 2 years		117,079	2	(532)	116,549
Total available-for-sale securities		\$192,442	\$8	\$(651)	\$191,799





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The following is a summary of short-term marketable securities classified as available-for-sale as of December 31, 2014 (in thousands):

	Cost	Unrealized Gains	Unrealized Losses	Estimated Fair Value
Marketable Equity Securities:				
Common Stock	\$5,160	\$4,499	\$(103 )	\$9,556
Total available-for-sale securities	\$5,160	\$4,499	\$(103 )	\$9,556

During 2015, 2014 and 2013, the Company recognized a loss of \$0.3 million, a gain of \$2.3 million and a gain of \$0.4 million on marketable securities, respectively. The Company had proceeds from the sale or maturity of marketable securities of \$10.0 million, \$6.5 million and \$4.4 million for 2015, 2014 and 2013, respectively. For the year ended December 31, 2015 the Company reclassified \$0.3 million from Other Comprehensive Income (Loss) to the Statement of Operations

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.

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 December 31, 2015 and 2014, the Company believed the cost basis for available-for-sale investments were recoverable in all material respects and there were no investments in an unrealized loss position for longer than 12 months.

**NOTE 5. DERIVATIVE FINANCIAL INSTRUMENTS**

Since 2013, the Company has issued 5 tranches of common stock purchase warrants to secure financing, remediate covenant violations related to the Credit Facility (See Note. 10) and provide consideration for Credit Facility amendments.

The Company accounts for derivative financial instruments in accordance with ASC 815-40, "Derivative and Hedging – Contracts in Entity's Own Equity" ("ASC 815-40"), instruments which do not have fixed settlement provisions are deemed to be derivative instruments. The Company's warrants are classified as liability instruments due to an anti-dilution provision that provides for a reduction to the exercise price of the warrants if the Company issues additional equity or equity linked instruments in the future at an effective price per share less than the exercise price then in effect.

**Issuances****2015**

On January 12, 2015, the Company entered into Amendment No. 3 to the Credit Facility discussed in Note 8, in which the Company obtained a commitment letter from Athyrium Capital Management, LLC and Perceptive Credit Opportunities Fund, LP (collectively, the "Lenders"), the Company's existing lenders, providing a commitment for a senior secured incremental term loan under the Company's existing term loan facility in an aggregate principal amount of \$30 million, which could have been drawn down at the Company's option to finance the acquisition of the assets of Asklepiion Pharmaceuticals, LLC.

As consideration for the commitment letter for the Incremental Loan, the Company made a cash payment to the Lenders and issued the Lenders warrants initially exercisable to purchase up to an aggregate of 125,000 shares of the Company's common stock. The Company recorded \$1.05 million of interest expense related to the warrants upon issuance.

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The Company calculated the fair value of the warrants using the Monte Carlo Simulation utilizing the following assumptions as of the grant date of the warrants:

Risk free rate	1.39	%
Expected volatility	85	%
Expected life (in years), represents the weighted average period until next liquidity event	0.3	
Expected dividend yield	—	
Exercise Price	\$13.25	

2014

In connection with the execution of the Credit Facility, the Company issued warrants to the lenders under the Credit Facility, initially exercisable to purchase up to an aggregate of 337,500 shares of common stock of the Company. The Warrants will be exercisable in whole or in part, at an initial exercise price per share of \$12.76 per share, which is subject to weighted-average anti-dilution protections. The Warrants may be exercised at any time upon the election of the holder, beginning on the date of issuance and ending on the fifth anniversary of the date of issuance.

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The total grant date fair value of the Warrants was \$2.5 million, was recorded as a derivative liability, and is included in the debt discount to the Note Payable in the consolidated balance sheets.

The Company calculated the fair value of the warrants using the Binomial Lattice pricing model using the following assumptions as of the grant date of the Warrants:

Risk free rate	1.62	%
Expected volatility	85	%
Expected life (in years), represents the weighted average period until next liquidity event	0.36	
Expected dividend yield	—	
Exercise Price	\$12.76	

On November 13, 2014, the Company entered into Amendment No. 2 to the Credit Facility which allowed the Company to be in compliance with certain covenants as of September 30, 2014. In addition certain covenants related to the 4<sup>th</sup> quarter of fiscal 2014 and 2015 were amended. As compensation for Amendment No. 2, the Company agreed to issue additional warrants to the lenders, initially exercisable to purchase an aggregate of 300,000 shares of common stock of the Company which were valued at \$2.2 million as of November 13, 2014, with an exercise price of \$9.96 per share, and was recorded in change in fair value of derivative instruments in the 2014 consolidated statements of operations.

**Re-measurement**

The warrants are re-measured at each balance sheet date based on estimated fair value. Changes in estimated fair value are recorded as non-cash valuation adjustments within other income (expenses) in the Company's accompanying consolidated statements of operations. The Company recorded a loss on a change in the estimated fair value of warrants of \$33.3 million, \$23.8 million, and \$10.1 million during the years ended December 31, 2015, 2014 and 2013, respectively.

The Company calculated the fair value of the warrants using the Monte Carlo Simulation as of December 31, 2015 and the Binomial Lattice options pricing model as of December 31, 2014, using the following assumptions:

	As of		
	December 31, 2015	December 31, 2014	
Fair value of common stock	\$19.29	\$12.24	
Expected life (in years), represents the weighted average period until next liquidity event	n/a**	0.33 years	
Remaining Life (in years) of the Warrants	2.1 – 4.0 years	3.1 – 4.9 years	
Risk-free interest rate	1.11 – 1.59%	1.13 – 1.69%	
Expected volatility	75 – 85%	85	%
Dividend yield	—	% —	%

\*\*There are no liquidity events expected within the life of the outstanding warrants.

Expected volatility is based on analysis of the Company's volatility, as well as the volatilities of guideline companies. 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.

The following tables presents the Company's derivative warrant issuances and balances outstanding during the years ended December 31, 2015 and 2014:

	Warrants	Weighted Average Exercise Price	Grant Date Fair Value
Outstanding at December 31, 2013	4,782,249	\$5.04	\$3.13
Issued	637,500	11.44	6.49
Canceled	—	—	—
Exercised	1,998,394	4.70	3.05
Outstanding at December 31, 2014	3,421,355	\$6.43	\$3.79
Issued	125,000	13.25	8.40
Canceled	—	—	—

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Exercised	880,807	5.35	3.23
Outstanding at December 31, 2015	2,665,548	\$7.05	\$4.20

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The following information applies to derivative warrants outstanding at December 31, 2015:

Exercise Price	Number of Warrants	Weighted Average Remaining Contractual Life (years)	Number Exercisable
\$3.60	660,036	2.12	660,036
\$6.00	1,243,012	2.62	1,243,012
\$12.76	337,500	3.5	337,500
\$9.96	300,000	3.87	300,000
\$13.25	125,000	4.03	125,000

The total intrinsic value of derivative warrants outstanding and exercisable as of December 31, 2015 is \$32.6 million. The Company's closing stock price was \$19.29 on December 31, 2015.

**NOTE 6. 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.

In estimating the fair value of the Company's derivative liabilities, the Company used the Monte Carlo Simulation as of December 31, 2015 and the Binomial Lattice options pricing model as of December 31, 2014. Based on the fair value hierarchy, the Company classified the derivative liability within Level 3.

In estimating the fair value of the Company's contingent consideration, the Company used the comparable uncontrolled transaction ("CUT") 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, notes receivable, deposits on license agreements, and accounts payable, due to their short term nature. The following table presents the Company's asset and liabilities that are measured and recognized at fair value on a recurring basis classified under the appropriate level of the fair value hierarchy as of December 31, 2015 (in thousands):

	As of December, 2015	Fair Value Hierarchy at December 31, 2015		
		Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)
Asset:				
Marketable securities, available-for-sale	\$ 191,799	\$—	\$ 191,799	\$—
Liabilities:				
Derivative liability related to warrants	\$ 38,810	\$—	\$—	\$38,810
Business combination-related contingent consideration	\$ 59,021	\$—	\$—	\$59,021

The following table presents the Company's asset and liabilities that are measured and recognized at fair value on a recurring basis classified under the appropriate level of the fair value hierarchy as of December 31, 2014 (in

thousands):

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	As of December, 2014	Fair Value Hierarchy at December 31, 2014		
		Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)
Asset:				
Marketable securities, available-for-sale	\$ 9,556	\$9,556	\$ —	\$—
Liabilities:				
Derivative liability related to warrants	\$ 27,990	\$—	\$ —	\$27,990
Business combination-related contingent consideration	\$ 11,637	\$—	\$ —	\$11,637

The following table sets forth a summary of changes in the estimated fair value of the Company's Level 3 derivative liability for the period from January 1, 2015 through December 31, 2015:

	Fair Value Measurements of Common Stock Warrants Using Significant Unobservable Inputs (Level 3)
Balance at January 1, 2015	\$ 27,990
Issuance of common stock warrants	1,050
Reclassification of derivative liability to equity upon exercise of warrants	(23,537 )
Change in estimated fair value of liability classified warrants	33,307
Balance at December 31, 2015	\$ 38,810

The following table sets forth a summary of changes in the estimated fair value of the Company's Level 3 derivative liability for the period from January 1, 2014 through December 31, 2014:

	Fair Value Measurements of of Common Stock Warrants Using Significant Unobservable Inputs (Level 3)
Balance at January 1, 2014	\$25,037
Issuance of common stock warrants	2,531
Reclassification of derivative liability to equity upon exercise of warrants	(23,364 )
Change in estimated fair value of liability classified warrants	23,786
Balance at December 31, 2014	\$27,990

A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. At each reporting period, the Company performs a detailed analysis of the assets and liabilities that are subject to ASC 820.

The following table sets forth a summary of changes in the estimated business combination-related contingent consideration for the period from January 1, 2015 through December 31, 2015:

Fair Value  
Measurements  
of  
Acquisition-Related  
Contingent

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	Consideration (Level 3)	
Balance at January 1, 2015	\$11,637	
Present value of contingent consideration related to Cholbam acquisition, upon acquisition	39,107	
Increase from revaluation of contingent consideration	13,778	
Decrease of contingent consideration, asset divestiture	(604	)
Contractual Payments	(3,938	)
Contractual Payments accrued at December 31, 2015	(959	)
Balance at December 31, 2015	\$59,021	

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The following table sets forth a summary of changes in the estimated acquisition-related contingent consideration for the period from January 1, 2014 through December 31, 2014:

	Fair Value Measurements of Acquisition-Related Contingent Consideration
Balance at January 1, 2014	\$—
Present value of contingent consideration related to Manchester acquisition, upon acquisition	12,800
Contractual Payments	(1,163 )
Balance at December 31, 2014	\$11,637

## NOTE 7. INTANGIBLE ASSETS

## Amortizable intangible assets

## Ligand License Agreement

In fiscal 2013, the Company entered into an agreement with Ligand Pharmaceuticals Incorporated for a worldwide sublicense for \$2.5 million to develop, manufacture and commercialize a drug technology compound including RE-021 or sparsentan (the “Ligand License Agreement”). The cost of the Ligand License Agreement, which is presented net of amortization in the accompanying consolidated balance sheet in intangible assets, net, is being amortized to research and development on a straight-line basis through September 30, 2023. As consideration for the license, we are required to make substantial payments upon the achievement of certain milestones, totaling up to \$105.5 million. Should we commercialize sparsentan or any products containing related compounds, we will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of all such products.

In September 2015, the license agreement was amended to facilitate sub-licensing in Asia-Pacific. As consideration for the amendment the Company paid \$1.0 million. The Company has \$3.3 million in intangibles related the Ligand license agreement as December 31, 2015.

## Carbetocin Technology

In September 2015, the Company wrote-off the entire value of intangible assets related to Carbetocin. The write-off was deemed appropriate as the Company elected not to pursue any internal development of the asset and attempts to divest it were unsuccessful. The total charge of \$4.7 million was included in operating expenses on the consolidated statement of operations and comprehensive income (loss).

## Manchester Pharmaceuticals LLC

The Company acquired intangible assets with finite lives related to the Chenodal product rights, trade names, and customer relationships with the values of \$71.4 million, \$0.2 million, and \$0.4 million, respectively. The useful lives related to the acquired product rights, trade names, and customer relationships are expected to be approximately 16, 1 and, 10 years, respectively. Amortization of product rights, trade names and customer relationships are being recorded in selling, general and administrative expense over their respective lives.

In 2015, the Company divested the assets related to Vecamyl, valued at \$3.6 million, to Turing Pharmaceuticals. The remaining product rights from the Manchester business combination relate to Chenodal and are \$67.8 million as of December 31, 2015.

## Thiola License Agreement

In 2014, the Company entered into a license agreement with Mission Pharmacal, in which the Company obtained an exclusive, royalty-bearing license to market, sell and commercialize Thiola (Tiopronin) in the United States and Canada, and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola. The initial term of the license is 10 years and will automatically renew thereafter for periods of one year.

The Company paid Mission an up-front license fee of \$3 million and will pay guaranteed minimum royalties during each calendar year the greater of \$2 million or twenty percent (20%) of the Company’s net sales of Thiola through June 30, 2024. As of December 31, 2015, the present value of guaranteed minimum royalties payable is \$10.9 million using a discount rate of approximately 11% based on the Company’s current borrowing rate. As of December 31, 2015, the

guaranteed minimum royalty current and long term liability is approximately \$0.8 million and \$10.1 million, respectively, and is recorded as guaranteed minimum royalty in the consolidated balance sheet. The Company has capitalized \$24.1 million related to the Thiola asset which consists of the up-front license fee, professional fees, present value of the guaranteed minimum royalties and any additional payments through 2015 in excess of minimum royalties. There is 8.4 years remaining in the initial term of the license agreement.

**Cholbam (Kolbam) Asset Purchase**

On March 31, 2015, the Company completed its acquisition from Asklepiion of all worldwide rights, titles and ownership of Cholbam, including all related contracts, data assets, intellectual property, regulatory assets and the PRV. The Company capitalized \$75.9 million and approximately \$7.3 million for the US and International economic interest, respectively.

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Amortizable intangible assets as of December 31, 2015 (in thousands):

	Useful Life	As of December 31, 2015		Net Book Value
		Gross Carrying Amount	Accumulated Amortization	
Chenodal Product Rights	16	\$67,849	\$(7,489)	) \$60,360
Thiola License	10	24,133	(2,793)	) 21,340
Economic Interest - U.S. revenue Cholbam	10	75,900	(5,715)	) 70,185
Economic Interest - Int'l revenue Cholbam	10	7,336	(552)	) 6,784
Ligand License	11	3,300	(765)	) 2,535
Manchester Customer Relationships	10	403	(71)	) 332
Manchester Trade Name	1	175	(175)	) —
Total		\$179,096	\$(17,560)	) \$161,536

Amortizable intangible assets as of December 31, 2014 (in thousands):

	Useful Life	As of December 31, 2014		Net Book Value
		Gross Carrying Amount	Accumulated Amortization	
Chenodal and Vecamyl Product Rights	16	\$71,372	\$(3,420)	) \$67,952
Thiola License	10	15,049	(870)	) 14,179
Syntocinon License	20	5,000	(190)	) 4,810
Carbetocin Assets	10	5,568	(430)	) 5,138
Ligand License	11	2,300	(527)	) 1,773
Manchester Customer Relationships	10	403	(31)	) 372
Manchester Trade Name	1	175	(134)	) 41
Total		\$99,867	\$(5,602)	) \$94,265

The following table summarizes amortization expense for the twelve months ended December 31, 2015, 2014 and 2013 (in thousands):

	Twelve months ended December 31,		
	2015	2014	2013
Research and development	\$697	\$823	\$324
Selling, general and administrative	12,534	4,455	—
Total amortization expense	\$13,231	\$5,278	\$324

As of December 31, 2015, amortization expense for the next five years is expected to be as follows (in thousands):

2016	\$15,517
2017	15,474
2018	15,474
2019	15,474
2020	15,476
Thereafter	84,121
Total	\$161,536

As of December 31, 2015 the remaining weighted average period of amortization is 11.06 years.

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## NOTE 8. ACCRUED EXPENSES

Accrued expenses consist of the following at December 31, 2015 and 2014:

	December 31, 2015	December 31, 2014
Compensation related costs	\$7,143	\$8,163
Severance related costs	315	5,710
Research and development	4,281	3,720
License fee	—	3,000
Legal fees	882	1,208
Interest	259	2,318
Government rebate reserves	3,158	1,353
Selling, general and administrative	2,703	2,411
Royalty/contingent consideration	4,688	—
Miscellaneous	391	—
	\$23,820	\$27,883

## NOTE 9. RELATED PARTY TRANSACTIONS

In the second quarter of 2013, the Company, its then-current Chief Executive Officer and a related party, a former investor in the Company that was previously managed by the Company's then-current Chief Executive Officer, became party to a series of agreements to settle up to \$2.3 million of liabilities, which Company management believes are the primary obligation of the related party. The Company and the related party have entered into indemnification agreements whereby the related party agreed to defend and hold the Company harmless against all such obligations and amounts, whether paid or unpaid, arising from these agreements. Notwithstanding the indemnification, the Company recorded a \$2.3 million charge to operations for the year ended December 31, 2013 for the (a) \$2.2 million of cash consideration, and (b) 11,000 shares of common stock valued at \$0.1 million of non-cash consideration. The \$2.3 million is entirely paid as of the date of this filing. In addition, the then-current Chief Executive Officer also agreed to provide one of the counter parties with 47,128 shares of his common stock in the Company as a separate component of one of these settlement agreements. Accordingly, the Company does not believe it is required to record a liability for the share-based component of this specific agreement. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

Concurrent with the execution of such settlement agreements, the Company and the related party entered into promissory notes whereby the related party agreed to pay the Company the principal amount of \$2.3 million plus interest at an annualized rate of 5% as reimbursement of payments that the Company made to settle a portion of the agreements.

On October 13, 2014, the Company entered into a binding Summary Separation Proposal with its then-current Chief Executive Officer. Among other matters, the Summary Separation Proposal set forth a summary of the terms for the sale of the Company's Vecamyl, Syntocinon and ketamine licenses and assets to Turing Pharmaceuticals, a company controlled by the former Chief Executive Officer.

On January 9, 2015, the Company entered into a purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals the Sold Assets for a purchase price of \$1.0 million, and pursuant to which Turing Pharmaceuticals also assumed all future liabilities related to the Sold Assets.

On February 13, 2015, the Sellers entered into a purchase agreement with Waldun, pursuant to which the Sellers sold Waldun the Vecamyl Product Rights for a purchase price of \$0.7 million. Waldun in turn sold the Vecamyl Product Rights to Turing Pharmaceuticals. In connection therewith, on February 13, 2015, the Company, together with Manchester, entered into an asset purchase agreement with Turing Pharmaceuticals, pursuant to which the Company sold Turing Pharmaceuticals the Inventory for a purchase price of \$0.3 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Vecamyl Product Rights and the Inventory.

On February 13, 2015, the Company entered into an asset purchase agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals its Oxytocin Assets, including related inventory, for a purchase

price of \$1.1 million, and pursuant to which Turing Pharmaceuticals also assumed certain liabilities related to the Oxytocin Assets.

The total impact to the Statement of Operations and Comprehensive Income (Loss) related to the divestitures for 2015 was a gain of \$0.9 million.

NOTE 10. NOTES PAYABLE

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 million aggregate principal senior convertible notes due 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, beginning on November 15, 2014. The Notes mature on May 30, 2019

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unless earlier converted or repurchased in accordance with the terms. The aggregate carrying value of the Notes on their issuance was \$43 million, which was net of the \$3 million debt discount.

On June 30, 2014, the Company issued 401,047 shares of Common Stock to the holders of the Notes and such Noteholders granted the Company a release of certain claims they may have had in connection with the Company's sale of the Notes or certain statements made by the Company in connection with such sale. The Company recorded finance expense as other expense in the amount of \$4.7 million for the year ended December 31, 2014 based on the fair market value of the stock on the date of issuance in relation to the shares issued.

As of December 31, 2015 the fair value of a share of common stock was \$19.29, exceeding the initial conversion price per share. If the debt holders were to convert the Company would be required to issue 2,642,160 shares assuming that no fundamental change in the Company has occurred. The Company has reserved sufficient shares of its Common Stock to satisfy the conversion requirements related to the Notes. As of December 31, 2015, the convert value exceeded the carrying value by approximately \$7.1 million.

As of December 31, 2015 the fair value of the debt is impractical to estimate.

The net carrying amount of the Notes consists of the following (in thousands):

	As of December 31,	
	2015	2014
Aggregate principle amount of Notes	\$46,000	\$46,000
Unamortized debt discount	(2,098	) (2,712
	\$43,902	\$43,288

**Credit Facility**

In June 2014, the Company entered into a \$45 million Credit Agreement (“Credit Facility”) which was scheduled to mature on June 30, 2018 and bore interest at an annual rate of (i) the Adjusted LIBOR Rate (as such term was defined in the Credit Facility) plus 10.00% or (ii) in certain circumstances, the Base Rate (as such term was defined in the Credit Agreement) plus 9.00% and was payable quarterly. The Credit Facility contained certain financial and non-financial covenants.

In connection with the execution of the Credit Facility, the Company issued warrants (the “Warrants”) to the lenders under the Credit Facility, initially exercisable to purchase up to an aggregate of 337,500 shares of common stock of the Company. The Warrants will be exercisable in whole or in part, at an initial exercise price per share of \$12.76 per share, which is subject to weighted-average anti-dilution protections. The Warrants may be exercised at any time upon the election of the holder, beginning on the date of issuance and ending on the fifth anniversary of the date of issuance. The issuance of the Warrants was not registered under the Securities Act of 1933, as amended (the “Securities Act”), as such issuance was exempt from registration under Section 4(2) of the Securities Act.

The total grant date fair value of the Warrants was \$2.5 million, was recorded as a derivative liability, and was included in the debt discount to the Note Payable in the 2014 consolidated balance sheets.

The Company calculated the fair value of the warrants using the Binomial Lattice pricing model using the following assumptions as of the grant date of the Warrants:

Risk free rate	1.62	%
Expected volatility	85	%
Expected life (in years), represents the weighted average period until next liquidity event	0.36	
Expected dividend yield	—	
Exercise Price	\$12.76	

In November 2014, the Company entered into Amendment No. 2 (“Amendment No. 2”) to the Credit Facility which allowed the Company to be in compliance with certain covenants as of September 30, 2014. In addition certain covenants related to the fourth quarter of fiscal 2014 and 2015 were amended. As compensation for Amendment No. 2, the Company agreed to issue additional warrants to Athyrium Capital Management, LLC and Perceptive Credit Opportunities Fund, LP (collectively, the “Lenders”), initially exercisable to purchase an aggregate of 300,000 shares of common stock of the Company which were valued at \$2.2 million and recorded in change in fair value of derivative instruments in the 2014 consolidated statements of operations.

On January 12, 2015, the Company entered into Amendment No. 3 (“Amendment No. 3”) to the Credit Facility in which the Company obtained a commitment letter from the Lenders, providing a commitment for a senior secured incremental term loan under the Company’s existing term loan facility in an aggregate principal amount of \$30.0 million, which could have been drawn down at the Company’s option to finance the acquisition of the Cholbam assets from Asklepiion.

As consideration for Amendment No. 3, the Company made a \$0.6 million cash payment to the Lenders, recorded in finance expense in the consolidated statements of operations, and issued the Lenders warrants initially exercisable to purchase up to an aggregate of 125,000 shares of the Company’s common stock which were valued at \$1.1 million on January 12, 2015 and were recorded in interest expense in the consolidated statements of operations. Due to the closing of its public offering on March 24, 2015, the Company received cash proceeds of \$140.0 million, after deducting underwriting fees

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and other offering costs, which the Company used to make the \$27.0 million payment due to Asklepiion upon the closing of the Company's acquisition of the Cholbam assets, and as a result, the Company did not utilize the commitment from the Lenders.

On July 1, 2015, the Company paid off the Credit Facility in its entirety including a prepayment premium of \$2.3 million, and incurred an additional charge of \$4.2 million, included in other expenses on the Company's consolidated statement of operations and comprehensive income (loss), for the write-off of the debt discount and equity issuances for the Credit Facility

#### Note Payable - Manchester Pharmaceuticals, LLC

On March 26, 2014, upon the acquisition of Manchester, the Company entered into a note payable in the amount of \$33 million. The note is non-interest bearing and therefore the Company recorded the loan at present value of \$31.3 million using the effective interest rate of approximately 11%, which was the Company's current borrowing rate. The note was due and payable in three consecutive payments, each in the amount of \$11 million payable on June 26, 2014, September 26, 2014, and December 12, 2014 (the maturity date). On June 30, 2014, the Company paid off the note in its entirety. The Company accelerated interest expense in the amount of \$1.7 million for the difference between the present value of the loan, and the loan balance paid was recorded in interest income (expense), net for the year ended December 31, 2014.

Total interest expense, net, recognized for the years ended December 31, 2015, 2014 and 2013 was \$7.7 million, \$7.4 million and \$0.0, respectively.

#### NOTE 11. COMMITMENTS AND CONTINGENCIES

##### Leases and Sublease Agreements

###### California Office

###### San Diego Office

On September 8, 2014, the Company entered into a lease agreement for its corporate headquarters located in San Diego, California. The Company rents its office space for approximately \$540,000 per annum plus escalations. The lease began on October 1, 2014 and expires on December 31, 2017.

###### Carlsbad Office - Vacated

In October 2014, Retrophin ceased use of this facility and all employees moved into the new headquarters facility in San Diego California. As a result of vacating this location, the Company recorded a loss of \$170,811 in the year ended December 31, 2014. On March 27, 2015 the Company was able to sublease a portion of this facility for the remaining lease term. The Company is in a listing agreement with a broker to market the remaining Carlsbad space for sublease.

###### Massachusetts Office

On July 31, 2014, the Company entered into a sublease agreement for new office space located in Cambridge, Massachusetts. The Company rents its office space for approximately \$815,000 per annum. The sublease expires on December 31, 2016.

###### New York Office

On December 30, 2015, the Company amended the lease agreement for its offices in New York, New York to extend the lease term through November 2018 and is responsible for approximately \$550,000 per annum in rent plus escalations.

##### Contractual Commitments

The following table summarizes our principal contractual commitments, excluding open orders that support normal operations, as of December 31, 2015 (in thousands):

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating leases	\$2,671	\$1,026	\$1,645	\$—	\$—
Note payable	53,073	2,070	4,140	46,863	—
Sales support services	3,470	312	937	833	1,388
Product supply contracts	5,221	3,923	1,298	—	—
Purchase order commitments	596	258	338	—	—
	\$65,031	\$7,589	\$8,358	\$47,696	\$1,388



Legal Proceedings

On January 7, 2014, the Company sued Questcor Pharmaceuticals, Inc. (“Questcor”) in federal court in the Central District of California (Retrophin, Inc. v. Questcor Pharmaceuticals, Inc., Case No. SACV14-00026-JLS). The Company alleged that Questcor violated antitrust laws in connection with its acquisition of rights to the drug Synacthen, and sought injunctive relief and damages. The Company asserted claims under sections 1 and 2 of the Sherman Act, section 7 of the Clayton Act, California antitrust laws, and California’s unfair competition law. On June 4, 2015, pursuant to the terms of a Confidential Settlement Agreement and Release (the “Settlement Agreement”) the Company and Questcor filed a Stipulation of Dismissal, dismissing

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the Company's lawsuit against Questcor. Under the terms of the Settlement Agreement, Questcor paid the Company \$15.5 million, recorded as "Litigation Settlement Gain" in 2015, and the Company and Questcor granted a mutual release of all claims against the other.

On June 13, 2014, Charles Schwab & Co., Inc. ("Schwab") sued the Company, Standard Registrar and Transfer Company ("Standard"), Jackson Su ("Su"), and Chun Yi Huang ("Huang") in federal court in the Southern District of New York (Charles Schwab & Co. v. Retrophin, Inc., Case No. 14-cv-4294). Su and Huang also asserted cross-claims against the Company and Standard for alleged negligent misrepresentation premised upon an alleged failure to inform them of restrictions on the sale of their Company stock, and impleaded Katten Muchin Rosenman LLP as a third-party defendant. Schwab's claims have been dismissed with prejudice. On September 30, 2015, the Court dismissed Su and Huang's cross-claims and third party claims. The dismissal was with prejudice with respect to Su, but without prejudice with respect to Huang. Huang did not seek leave to re-plead his claims within the time set by the Court. Accordingly, on November 10, 2015, the Court ordered the case to be closed.

On September 19, 2014, purported shareholders of the Company sued Martin Shkreli, the Company's former Chief Executive Officer, in federal court in the Southern District of New York (Donoghue v. Retrophin, Inc., Case No. 14-cv-7640). The Company is a nominal defendant in this action. The plaintiffs sought, on behalf of the Company, disgorgement of short-swing profits from Mr. Shkreli under section 16(b) of the Securities Exchange Act of 1934 (15 U.S.C. 78(p)(b)). The Court has approved a settlement between the parties, under which Mr. Shkreli is obligated to pay \$2,025,000 to the Company and an additional \$625,000 to Plaintiffs to compensate them for their legal fees. Shkreli has defaulted on the judgment and the Company and the Plaintiffs are taking steps to collect it. The Company has not recorded anything related the judgment for 2015. Any related amounts received will be recorded against equity when collected.

On October 20, 2014, a purported shareholder of the Company filed a putative class action complaint in federal court in the Southern District of New York against the Company, Mr. Shkreli, Marc Panoff, and Jeffrey Paley (Kazanchyan v. Retrophin, Inc., Case No. 14-cv-8376). On December 16, 2014, a second, related complaint was filed in the Southern District of New York against the same defendants (Sandler v. Retrophin, Inc., Case No. 14-cv-9915). The complaints assert violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 in connection with defendants' public disclosures during the period from November 13, 2013 through September 30, 2014. In December 2014, plaintiff Kazanchyan filed a motion to appoint lead plaintiff, to approve lead counsel, and to consolidate the two related actions. On February 10, 2015, the Court consolidated the two actions, appointed lead plaintiff, and approved lead counsel. Lead plaintiff filed a consolidated amended complaint on March 4, 2015, which again named the Company, Mr. Shkreli, Mr. Panoff, and Mr. Paley as defendants, but which also named Steven Richardson, Stephen Aselage, and Cornelius Golding as additional defendants. On May 26, 2015, with the consent of the lead plaintiff, the court ordered that the claims against Mr. Paley be dismissed. The remaining defendants, including the Company, filed motions to dismiss the consolidated amended complaint, which were fully-briefed as of October 29, 2015. On December 1, 2015, counsel jointly informed the Court that the parties had reached a comprehensive settlement, subject to Court approval. On January 29, 2016, the parties filed motion for preliminary approval of the settlement and supporting papers, including a stipulation of settlement. On February 2, 2016, the Court preliminarily approved the settlement and scheduled a final approval hearing for June 10, 2016. Any amounts owed by the Company would be covered by Director and Officer Insurance.

In January 2015, the Company received a subpoena relating to a criminal investigation by the U.S. Attorney for the Eastern District of New York. The subpoena requested information regarding, among other things, the Company's relationship with Mr. Shkreli and individuals or entities that had been investors in investment funds previously managed by Mr. Shkreli. The Company has been informed that it is not a target of the U.S. Attorney's investigation, and is cooperating with the investigation. On December 17, 2015, an indictment against the Company's former Chief Executive Officer, Martin Shkreli, and its former outside counsel, Evan Greebel, was unsealed in the United States District Court for the Eastern District of New York. The Company has also been cooperating with a parallel investigation by the U.S. Securities and Exchange Commission (the "SEC"). On December 17, 2015, the SEC filed a civil complaint against Mr. Shkreli, Mr. Greebel, MSMB Capital Management LLC, and MSMB Healthcare Management LLC in the United States District Court for the Eastern District of New York.

On August 17, 2015, the Company filed a lawsuit in federal district court for the Southern District of New York against Martin 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 (Retrophin, Inc. v. Shkreli, 15-CV-06451(NRB)). On August 19, 2015, 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 has asserted counterclaims in the arbitration that are substantially similar to the claims it previously asserted in the federal lawsuit against Mr. Shkreli. The parties have selected an arbitration panel. On Mr. Shkreli's application, and with the Company's consent, the federal Court has granted a stay of the federal lawsuit pending a determination by the arbitration panel whether the Company's counterclaims will be litigated in the arbitration, as the Company is seeking. As of December 31, 2015 no accruals for loss contingencies have been recorded since these cases are neither probable nor reasonably estimable. From time to time the Company is involved in legal proceedings arising in the ordinary course of business. The Company believes there is no other litigation pending that could have, individually or in the aggregate, a material adverse effect on its results of operations or financial condition.

Under the Company's bylaws, current and former officers and directors may seek advancement for certain expenses, including attorneys' fees. The Company has recently received a number of significant requests for advancement, and is in discussions about how much of the amounts sought the Company is obligated to pay. In addition, for certain of these amounts, the Company's obligation to pay advancement is eligible for reimbursement under the Company's insurance policies. Therefore, the Company is unable at this time to estimate the amount of advancement currently sought from the Company that will ultimately be eligible for advancement, nor how much of the amounts eligible for advancement will be eligible for reimbursement under the Company's insurance policies, and whether or not the amounts could be material.

NOTE 12. STOCKHOLDERS' EQUITY / DEFICIT

Common Stock

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The Company is currently authorized to issue up to 100,000,000 shares of \$0.0001 par value common stock. All issued shares of common stock are entitled to vote on a 1 share/1 vote basis.

**Preferred Stock**

The Company is currently authorized to issue up to 20,000,000 shares of \$0.001 preferred stock, of which 1,000 shares are designated Class "A" Preferred shares, \$0.001 par value. Class A Preferred Shares are not entitled to interest, have certain liquidation preferences, special voting rights and other provisions. No Preferred Shares have been issued to date.

**Public Offering - 2015**

On March 24, 2015, the Company completed a public offering of 7,866,000 shares of common stock at a price of \$19.00 per share. We received net proceeds from the offering of \$140.0 million after deducting underwriting fees and other offering costs of \$9.5 million. The shares of common stock were offered by us pursuant to a shelf registration statement that was declared effective by the SEC on March 13, 2015.

**2014 Incentive Compensation Plan**

On May 9, 2014, the Company's stockholders approved the 2014 Incentive Compensation Plan (the "Plan"). The Plan authorizes the granting of stock options, stock appreciation rights, restricted stock and restricted stock units, deferred stock, performance units and annual incentive awards covering up to 3.0 million shares of the Company's common stock. In a special shareholder meeting held February 3, 2015, the Company's shareholders approved an incremental 1,928,000 shares of common stock and 230,000 restricted shares of common stock. These shares were granted to employees between February 24, 2014 and August 18, 2014.

**2015 Equity Incentive Plan**

On June 8, 2015, the Company's stockholders approved the 2015 Equity Incentive Plan (the "2015 Plan"). The plan is intended as the successor to and continuation of the Plan. Stockholders approved 1.4 million new shares to be issued under the 2015 Plan, in addition to 0.6 million unallocated shares remaining available for issuance under the Plan that were added to the 2015 Plan.

**Stock Options**

The fair values of stock option grants during the year ended December 31, 2015, 2014 and 2013 were calculated on the date of grant using the Black-Scholes option pricing model, except for options granted for market and revenue performance criteria. Compensation expense is recognized over the period of service, generally the vesting period. During the year ended December 31, 2015, 2,285,000 stock options were granted by the Company. The following weighted average assumptions were used in the Black-Scholes options pricing model to estimate the fair value of stock options for the specified reporting periods:

	Year Ended December 31, 2015		Year Ended December 31, 2014		Year Ended December 31, 2013	
Risk free rate	1.53	%	1.55	%	1.51	%
Expected volatility	83	%	85	%	102	%
Expected life (in years)	5.8		5.8		5.8	
Expected dividend yield	—		—		—	

The risk-free interest rate was based on rates established by the Federal Reserve. The Company's expected volatility was based on analysis of the Company's volatility, as well as the volatilities of guideline companies. The expected life of the Company's options was determined using the simplified method as a result of limited historical data regarding the Company's activity. The dividend yield is based upon the fact that the Company has not historically paid dividends, and does not expect to pay dividends in the foreseeable future.

The following table summarizes our stock option activity and related information for the year ended December 31, 2015:

Shares Underlying Options	Weighted Average		
	Exercise Price	Remaining Contractual	Aggregate Intrinsic Value (in thousands)

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			Term (in years)	
Exercisable at December 31, 2014	1,225,833	\$9.73	7.96	\$3,395
Outstanding at December 31, 2014	4,892,208	\$10.93	8.57	\$8,353
Granted	2,285,000	\$27.15	—	—
Forfeited and expired	(970,170	) 14.91	—	—
Exercised	(541,454	) 13.10	—	—
Outstanding at December 31, 2015	5,665,584	\$17.05	8.75	\$31,542
Exercisable at December 31, 2015	2,036,906	\$12.55	8.34	\$15,582

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The weighted average grant date fair value of options granted is \$19.02, \$8.56, and \$6.03 during the years ended December 31, 2015, 2014 and 2013, respectively. The aggregate intrinsic value for outstanding options is calculated as the difference between the exercise price of the underlying awards and the quoted price of the Company's common stock as of December 31, 2015 of \$19.29. Unrecognized compensation cost associated with unvested stock options amounts to \$47.4 million as of December 31, 2015, which will be expensed over a weighted average remaining vesting period of 1.9 years.

**Restricted Shares**

As of December 31, 2015, there was approximately \$7.1 million of unrecognized compensation cost related to restricted shares granted. This amount is expected to be recognized over a weighted average period of 1.8 years. Unvested restricted shares consist of the following as of December 31, 2015:

	Number of shares	Weighted Average Grant Date Fair Value
Unvested December 31, 2014	691,668	\$10.83
Granted	273,000	26.97
Vested	(478,334	) 11.56
Forfeited/cancelled	(56,668	) 13.97
Unvested December 31, 2015	429,666	\$20.38

**Share Based Compensation**

Total non-cash stock-based compensation expense consisted of the following for the years ended December 31, 2015 and 2014 (in thousands):

	Year Ended December 31,		
	2015	2014	2013
Selling, general and administrative expenses	\$16,483	\$10,940	\$2,651
Research and development expenses	9,417	4,960	259
Total	\$25,900	\$15,900	\$2,910

**Exercise of Warrants**

During the twelve months ended December 31, 2015, the Company issued 870,306 shares of common stock upon the exercise of warrants for cash received by the Company in the amount of \$4.5 million. The Company reclassified \$23.5 million derivative liability as equity for the value of these warrants on the date of exercise. The warrants were revalued immediately prior to exercise and the change in the fair value of \$2.8 million was recorded as other expense in the consolidated financial statements of the Company.

During the twelve months ended December 31, 2014, the Company issued 1,947,377 shares of common stock upon the exercise of warrants for cash received by the Company in the amount of \$8.4 million. The Company reclassified \$23.4 million derivative liability as equity for the value of these warrants on the date of exercise. The warrants were revalued immediately prior to exercise and the change in the fair value of the warrants was recorded as other expense in the consolidated financial statements of the Company.

**Treasury Stock**

In the fourth quarter of 2013, the Company repurchased 130,790 shares of its common stock for an aggregate purchase price of \$957,272. The Company currently recognizes such repurchased common stock as treasury stock.

During the year ended December 31, 2014, the Company repurchased 248,801 shares of its common stock for an aggregate purchase price of \$2.3 million. The Company recognizes repurchased common stock as treasury stock. In March 2015 the Company retired 379,591 shares of its common stock held as treasury stock. This was the entire holding of treasury stock. No other shares were repurchased during the year.

**NOTE 13. EARNINGS (LOSS) PER SHARE**

Basic earnings (loss) per share ("EPS") represent net income (loss) attributable to common shareholders divided by the weighted average number of common shares outstanding during the measurement period. Diluted EPS represents net income attributable to common shareholders divided by the weighted average number of common shares outstanding

during the measurement period while also giving effect to all potentially dilutive common shares that were outstanding during the period using the treasury stock method.

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Basic and diluted net EPS is calculated as follows (net income amounts are stated in thousands):

	For the year ended December 31,			2014			2013		
	2015	Net Income	EPS	Shares	Net Income	EPS	Shares	Net Income	EPS
Basic Earnings per Share	33,560,249	\$ 117,237	\$ 3.49	25,057,509	\$(110,938)	\$(4.43)	14,205,264	\$(34,625)	\$(2.44)
Convertible Debt	2,642,160	1,881	—	—	—	—	—	—	—
Restricted Stock	290,966	—	—	—	—	—	—	—	—
Stock Options	1,088,064	—	—	—	—	—	—	—	—
Dilutive Earnings per Share	37,581,439	\$ 119,118	\$ 3.17	25,057,509	\$(110,938)	\$(4.43)	14,205,264	\$(34,625)	\$(2.44)

For the years ended December 31, 2015, 2014 and 2013, the following shares were excluded because they were anti-dilutive:

	For the year ended December 31,		
	2015	2014	2013
Restricted Stock	22,069	—	—
Options	1,049,375	1,132,500	172,667
Warrants	2,665,548	3,083,855	4,462,426
Total Anti-Dilutive Shares	3,736,992	4,216,355	4,635,093

**NOTE 14. INCOME TAXES**

For financial reporting purposes, net income before income taxes includes the following components (in thousands):

	Year Ended December 31,		
	2015	2014	2013
United States	\$ 107,038	\$(112,558)	\$(34,549)
Foreign	(1,571)	(840)	—
Total	\$ 105,467	\$(113,398)	\$(34,549)

For the year ending December 31, 2015, the Company became a taxpayer and is therefore required to pay its estimated Federal and State income taxes quarterly throughout the year. The taxes paid were based upon estimated taxable income, which differed from our final results, so we have prepaid tax of \$8.1 million, which we will apply to our 2016 quarterly tax estimates.

The components of the provision (benefit) for income taxes, in the consolidated statement of operations are as follows (in thousands):

	2015	2014	2013
Current			
Federal	\$ 2,094	\$—	\$—
State	1,709	—	—
	3,803	—	—
Deferred			
Federal	(8,296)	(1,886)	(6,293)
State	(7,277)	(574)	(3,435)
	(15,573)	(2,460)	(9,728)
Change in valuation allowance	—	—	9,804
Total tax provision (benefit)	\$(11,770)	\$(2,460)	\$ 76

During the year ended December 31, 2015, in connection with the acquisition of Cholbam, the Company recorded a deferred tax liability of \$39.9 million. Based on the fact that the reversal of the deferred tax liability is viewed as a source of future income pursuant to ASC 740, the Company was able to reduce its existing valuation allowance by \$39.9 million. The deferred tax liabilities supporting the ability to realize the deferred tax assets in the above acquisition will reverse in the same period, are in the same jurisdiction and are of the same character as the temporary



differences that gave rise to those deferred tax assets.

Additionally, during the year ended December 31, 2015, the Company recorded tax expense of \$28.1 million primarily relating to current and deferred tax expense accrued on the sale of Priority Review Voucher (“PRV”), partially offset by release of valuation allowance pursuant to the utilization of net operating loss carry-forwards primarily related to the PRV sale.

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The following is a reconciliation of the statutory federal income tax rate to the Company's effective tax rate expressed as a percentage of income (loss) before income taxes:

	2015	2014	2013	
Statutory rate - federal	35.00	% (35.00)	)% (35.00)	)%
State taxes, net of federal benefit	1.53	% (6.77)	)% (6.70)	)%
Change in FV of derivative liability (warrants)	10.89	% 7.40	% 10.46	%
Stock Based Compensation	—	% 5.51	% 2.30	%
Bargain purchase gain	(16.04	)%		
Other permanent differences	3.68	%		
Tax credits	(7.85	)%		
Return to provision adjustments and other true-ups	(10.40	)%		
Other	(0.79	)%	—	% 0.17
Change in valuation allowance	(27.02	)%	26.63	% 29.00
Income tax provision (benefit)	(11.00	)%	(2.23	)% 0.23

The significant components of the Company's deferred tax assets and liabilities as of December 31, 2015 and 2014 are as follows (in thousands):

	2015	2014
<b>Deferred Tax Assets:</b>		
Net operating loss	\$2,870	\$42,280
Contingent consideration	21,575	
Other accrued expenses	3,160	
Stock based compensation	9,484	
Other	—	1,427
	37,089	43,707
<b>Deferred Tax Liabilities:</b>		
Intangible assets	(25,124	) (7,830
Deferred gain on installment sale	(29,095	)
Tax basis depreciation less than book depreciation	(218	)
	(54,437	) (7,830
Net deferred tax assets before valuation allowance	(17,348	) 35,877
Valuation allowance	(6,980	) (36,018
Total deferred tax liability	\$(24,328	) \$(141

At December 31, 2015, the Company has available unused U.S. federal net operating loss ("NOL") carryforwards of \$8.2 million and a minimal amount of state NOL carryforwards. The U.S. federal NOL carryforwards will expire beginning in 2030. The Company has international subsidiaries whose operations are not material for the year ended December 31, 2015.

The Company's utilization of the net operating loss carryforwards may be subject to annual limitations due to the ownership change limitations provided by Internal Revenue Code ("IRC") Section 382 and similar state provisions. Pursuant to IRC Section 382, the annual use of the Company's net operating loss carryforwards may be limited in the event a cumulative change in ownership of more than 50% occurs within a three-year period. The annual limitations may result in the expiration of net operating losses prior to utilization. The annual limitation is determined based upon the fair market value of the Company as of the date of such ownership change. Based on the value of the Company at all relevant dates, the computed annual limitation that would result from an ownership change of the Company is not expected to prevent us from utilizing the majority of our remaining net operating losses prior to their expiration. The Company accounts for uncertain tax benefits in accordance with the provisions of ASC 740-10 of the Accounting for Uncertainty in Income Taxes. Of the total unrecognized tax benefits at December 31, 2015, approximately \$1.8 million was recorded as a reduction to deferred tax assets. If recognized, \$1.8 million of unrecognized tax benefits would affect the Company's effective tax rate. Additionally, as of December 31, 2015 and 2014 the Company had

recorded an indemnification asset with a corresponding liability in the amount of \$1.5 million for an uncertain tax position related to the acquisition of Manchester Pharmaceuticals, LLC. The Company is indemnified with respect to the liability.

The Company does not anticipate that the amount of unrecognized tax benefits as of December 31, 2015 will change materially within the 12 month period following December 31, 2015.

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A reconciliation of the Company's unrecognized tax benefits for the years 2015 and 2014 is provided in the following table (in thousands):

	2015	2014
Balance as of January 1:	\$1,500	\$—
Increase in current period positions	1,424	1,500
Increase in prior period positions	400	—
Balance as of December 31:	\$3,324	\$1,500

The Company files income tax returns in the U.S. federal jurisdiction and various state and local jurisdictions. The Company's income tax returns are open to examination by federal, state and foreign tax authorities, generally for the years ended December 31, 2012 and later.

The Company's policy is to record estimated interest and penalties related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision. During the years ended 2015, 2014 and 2013, the Company did not recognize any interest or penalties in its statements of operations and there were no accruals recorded for interest or penalties at December 31, 2015 and 2014.

**NOTE 15. INVESTIGATIONAL MATTERS****Investigation and Impact on Financial Statements**

In September 2014, the Company's board of directors requested that its outside legal counsel conduct an investigation into various matters related to the former Chief Executive Officer of the Company. In January 2015, our board of directors appointed an Oversight Committee to oversee and direct the investigation and make findings and decisions related to the investigation. As a result of the investigation, the Oversight Committee determined that, throughout 2013 and 2014, the former Chief Executive Officer engaged in a series of transactions (the "Prior Transactions"), which involved individuals and entities that had been investors in investment funds previously managed by the former Chief Executive Officer (the "MSMB Entities"), pursuant to which assets of the Company were misappropriated.

As a result of the Prior Transactions the financial statements contained in the Company's Form 10-Q for the three months ended September 30, 2013 (the "2013 Q3 Form 10-Q"), the Company's Form 10-K for the year ended December 31, 2013 (the "2013 Form 10-K") and the Company's Forms 10-Q for the quarters ended March 31, 2014, June 30, 2014 and September 30, 2014 (the "2014 Forms 10-Q") contained errors related to the reporting of certain consulting agreements entered into as part of the Prior Transactions, the predominant purpose of which appears to have been to settle and release claims against the MSMB Entities or the former Chief Executive Officer personally. On February 19, 2015, our board of directors concluded that as a result of the errors related to such consulting agreements, the financial statements contained in the 2013 Q3 Form 10-Q and the 2013 Form 10-K should no longer be relied upon. Accordingly, the Quarterly Report on Form 10-Q for the quarter ending September 30, 2013 and the Annual Report on Form 10-K for the year ended December 31, 2013 were amended and filed with the SEC in July 2015.

**Stock Option Accounting**

The Company held a Special Meeting of Stockholders on February 3, 2015, at which its stockholders voted to approve a proposal ratifying the prior issuance of stock options to purchase 1,928,000 shares of common stock and 230,000 restricted shares of common stock granted to employees between February 24, 2014 and August 18, 2014 (the "Ratified Equity Grants"). The 2014 Forms 10-Q contained errors related to the non-cash compensation expense recognized in connection with the Ratified Equity Grants, because the grant/measurement date of the Ratified Equity Grants for financial accounting purposes did not occur until their ratification in 2015.

The Company previously accounted for the Ratified Equity Awards as if a grant/measurement date for financial accounting purposes had occurred upon their issuance date, and recognized compensation expense for such Ratified Equity Awards based on the grant/measurement date value, which is amortized ratably to compensation expense and additional paid-in capital over the applicable service periods. The Company should have accounted for the Ratified Equity Awards as equity grants without a grant/measurement date, which are accounted for as "liability awards", with compensation expense and an offsetting compensation liability recorded over the term of the award, and the liability award revalued at each reporting period based on changes in the Company's stock price until it is ratified.

The Company believes that the errors in the 2014 Forms 10-Q related to the non-cash compensation expense recognized in connection with the Ratified Equity Grants do not cause the financial statements included within the 2014 Forms 10-Q to be misleading, and therefore such financial statements can still be relied upon. The Company corrected such errors, including any related disclosures, in its 2014 Annual Report on Form 10-K, and restated those quarters in 2014 Form 10-Q filings.

On February 27, 2015, the Company received a Public Letter of Reprimand from NASDAQ (the “Letter of Reprimand”), in accordance with Nasdaq Listing Rule 5810(c)(4). The Letter of Reprimand communicates NASDAQ’s belief that the interests of the Company’s shareholders were not materially adversely affected by the matters described above, and while not having been cured, the violation described above was remediated to the extent possible. Accordingly, NASDAQ does not believe that the delisting of the Company’s securities is an appropriate sanction, but rather, the circumstances warranted the issuance of the Letter of Reprimand. The issuance of the Letter of Reprimand completes NASDAQ’s review of the matters described above.

NOTE 16. SEVERANCE AGREEMENTS

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On September 15, 2014, the Company entered into a separation agreement and release (the “Separation Agreement”) with Marc Panoff, the Company’s Chief Financial Officer, pursuant to which Mr. Panoff’s employment with the Company was terminated effective as of February 28, 2015. Under the terms of the Separation Agreement, Mr. Panoff will be entitled to receive: (i) severance payments equal to six months of his current base salary; (ii) 100% of his target bonus for 2014; (iii) accelerated vesting of 81,333 shares of restricted common stock of the Company; and (iv) benefits under the Company’s benefit plans, subject to the terms of each such plan. In conjunction with the Separation Agreement, the Company had initially recorded and accrued \$0.1 million of severance expense through September 30, 2014 in connection with Mr. Panoff’s severance which was to be expensed ratably over the service period from September 15, 2014 through February 28, 2015. During the 4<sup>th</sup> quarter of 2014, the Company determined that Mr. Panoff’s service to the Company was substantially completed prior to December 31, 2014 and as a result recorded the remaining unamortized severance expense related to his separation agreement of \$1.1 million in the 4<sup>th</sup> quarter of fiscal 2014 in selling, general and administrative in the consolidated statements of operations. Mr. Panoff’s target bonus which was included as part of his severance agreement was recognized ratably over the course of the fiscal year ended December 31, 2014.

On October 13, 2014, Martin Shkreli resigned as a member of the Board and as an employee of the Company, and from any and all other positions that he held with the Company. On October 13, 2014, the Company entered into a resignation letter with Mr. Shkreli (“Separation Agreement”). As part of Mr. Shkreli’s Separation Agreement, Mr. Shkreli has been receiving cash severance, unpaid bonus and health insurance coverage, 12 months of continued vesting of time based stock options and no vesting of performance based stock options. Pursuant to the Separation Agreement, Mr. Shkreli’s market and performance based stock options have been forfeited. As a result, the Company recorded compensation expense in the amount of \$0.5 million relating to Mr. Shkreli’s cash severance, unpaid bonus and health insurance coverage and compensation expense of \$1.1 million related to the accelerated vesting of Mr. Shkreli’s time based stock options.

On October 13, 2014, the Company signed a Letter of Intent for the terms for the sale of the Company’s Vecamyl, Syntocinon and ketamine licenses and assets to Turing Pharmaceuticals AG (“Turing Pharmaceuticals”), which includes an up-front payment to the Company of \$3.0 million and the assumption of certain liabilities including license fees and royalties (the “Sale Transaction”). Martin Shkreli, the Company’s former Chief Executive Officer and Director, is the Chief Executive Officer of Turing Pharmaceuticals. The closing of the Sale Transaction was subject to various conditions, including the negotiation and execution of a binding definitive agreement between the Company and Turing Pharmaceuticals and the receipt of necessary third party consents. In connection with the Letter of Intent with Martin Shkreli, the Company recorded severance expense and accrued severance expense of \$2.9 million as of and for the year ended December 31, 2014 which is the difference between of the net book value of the assets to be sold, the \$3.0 million expected upfront payment, and \$3.0 million of liabilities expected to be assumed.

As both transactions were contemplated simultaneously, they were both considered in calculating the respective severance expense related to Mr. Shkreli’s termination. The full amount of the severance was recorded as of September 30, 2014 as that was the date that the Board replaced Martin Shkreli as Chief Executive Officer of the Company until a formal separation agreement could be finalized. As of September 30, 2014, it was deemed to be probable and estimable that Mr. Shkreli would enter into a Separation Agreement that would entitle him to severance benefits. Therefore the estimated severance that was booked as of the end of the third quarter is based on the best estimate currently available and the full severance amount was recorded as of September 30, 2014 as Mr. Shkreli was not required to perform any future service for the Company. For the year ended December 31, 2014, the Company recorded a total of \$4.5 million severance expense in connection with Mr. Shkreli’s Separation Agreement which has been recorded in selling, general and administrative expenses in the consolidated statements of operations.

On January 9, 2015, the Company entered into an Asset Purchase Agreement (the “Purchase Agreement”) with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals its ketamine licenses and assets (the “Ketamine Assets”) for a purchase price of \$1.0 million. Turing Pharmaceuticals will also assume all future liabilities related to the Ketamine Assets.

On February 13, 2015, Retrophin, Inc., its wholly-owned subsidiary Manchester and its other wholly-owned subsidiary Retrophin Therapeutics International, LLC (collectively, the “Sellers”), entered into a Purchase Agreement

with Waldun, pursuant to which the Sellers sold Waldun their product rights to mecamlamine hydrochloride (also referred to as Vecamyl) (the "Vecamyl Product Rights") for a purchase price of \$0.7 million. Waldun in turn sold the Vecamyl Product Rights to Turing Pharmaceuticals. In connection therewith, on February 13, 2015, the Company and Manchester entered into an Asset Purchase Agreement with Turing Pharmaceuticals, pursuant to which the Company and Manchester sold Turing Pharmaceuticals their Vecamyl inventory for a purchase price of \$0.3 million. Turing Pharmaceuticals will also assume certain liabilities related to the Vecamyl Product Rights and Inventory. Additionally, on February 13, 2015, the Company entered into an Asset Purchase Agreement with Turing Pharmaceuticals pursuant to which the Company sold Turing Pharmaceuticals its Syntocinon licenses and assets, including related inventory, for a purchase price of \$1.1 million. Turing Pharmaceuticals will also assume certain liabilities related to the Syntocinon licenses and assets.

**NOTE 17. SUBSEQUENT EVENTS**

On January 27, 2016 the Company reached a settlement with its former counsel, Katten Munchin Rosenman, LLP (Katten). Under the terms of the agreement, the parties exchanged general releases, including Katten's claim for \$3.0 million in unpaid invoices for Katten legal services incurred during the 2012-2014. The Company will record a \$3.0 million reversal of the obligation of the Katten legal invoices during the 1st quarter of 2016.

On October 20, 2014, a purported shareholder of the Company filed a putative class action complaint in federal court in the Southern District of New York (Kazanchyan v. Retrophin, Inc., Case No. 14-cv-8376). On December 16, 2014, a second, related complaint was filed in the Southern District of New York (Sandler v. Retrophin, Inc., Case No. 14-cv-9915). On February 10, 2015, the Court consolidated the two actions. On December 1, 2015, counsel jointly informed the Court that the parties had reached a comprehensive settlement, subject to Court approval. On January 29, 2016, the parties filed motion for preliminary approval of the settlement and supporting papers, including a stipulation of settlement. On February 2, 2016, the

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Court preliminarily approved the settlement of \$3.0 million and scheduled a final approval hearing for June 10, 2016. The \$3.0 million will be covered by the Company's insurance.

On February 17, 2016 Retrophin sued MSMB Capital Management LLC, MSMB Capital Management LP, MSMB Healthcare LP, MSMB Healthcare Investors, LLC, and MSMB Healthcare Management, LLC (the "MSMB Funds"), a series of hedge fund entities founded by Retrophin's former Chief Executive Officer Martin Shkreli to collect on five promissory notes between the MSMB Funds and Retrophin. The Company asked a judge to order the fund to repay \$2.18 million in principal and 5 percent interest. The case is Retrophin Inc. v. MSMB Capital Management LLC, 650813/2016, New York State Supreme Court, New York County (Manhattan).

On February 24, 2016, the Company announced that the European Commission has granted orphan drug designation to RE-024, the Company's novel investigational phosphopantothenate replacement therapy for pantothenate kinase-associated neurodegeneration (PKAN), a rare and life-threatening genetic disorder with no approved treatment option.

## NOTE 18. QUARTERLY FINANCIAL INFORMATION (UNAUDITED)

The following table presents selected Consolidated Statements of Operations data for each quarter for the fiscal year ended December 31, 2015 and 2014.

	For the year ended December 31,			
	Fourth Quarter	Third Quarter	Second Quarter	First Quarter
2015:				
Net product sales	\$30,447	\$28,005	\$24,068	\$17,372
Total operating expenses	45,651	48,501	31,012	25,476
Operating loss	(15,204	) (20,496	) (6,944	) (8,104
Total other income (expense), net	2,210	164,835	(18,568	) 7,738
Income (loss) before provision for income taxes	(12,994	) 144,339	(25,512	) (366
Income tax benefit(provision)	10,525	(38,761	) (15	) 40,021
Net income (loss)	\$(2,469	) \$105,578	\$(25,527	) \$39,655
Per Share Data:				
Net income (loss) per common share, basic	\$(0.07	) \$2.95	\$(0.73	) \$1.46
Net income (loss) per common share, diluted	\$(0.14	) \$1.78	\$(0.73	) \$1.32
2014:				
Net product sales	\$14,085	\$8,348	\$5,742	\$28
Total operating expenses	32,782	30,215	22,924	22,090
Operating loss	(18,697	) (21,867	) (17,182	) (22,062
Total other income (expense), net	(10,330	) 3,887	26,462	(53,609
Income (loss) before provision for income taxes	(29,027	) (17,980	) 9,280	(75,671
Income tax benefit(provision)	—	—	2,525	(65
Net income (loss)	\$(29,027	) \$(17,980	) \$11,805	\$(75,736
Per Share Data:				
Net income (loss) per common share, basic	\$(1.10	) \$(0.67	) \$0.46	\$(3.25
Net income (loss) per common share, diluted	\$(1.10	) \$(0.84	) \$(0.77	) \$(3.25