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

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**FORM 10-Q**

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(Mark One)

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

For the quarterly period ended September 30, 2015

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

**RETROPHIN, INC.**

(Exact name of registrant as specified in its charter)

<u>Delaware</u> (State or other jurisdiction of incorporation or organization)	<u>001-36257</u> (Commission File No.)	<u>27-4842691</u> (I.R.S. Employer Identification No.)
 12255 El Camino Real, Suite 250, San Diego, CA 92130 (Address of Principal Executive Offices)		
 (760) 260-8600 (Registrant's Telephone number including area code)		

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>

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

The number of shares of outstanding common stock, par value \$0.0001 per share, of the Registrant as of November 2, 2015 was 36,148,930.

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## RETROPHIN, INC.

Form 10-Q  
September 30, 2015

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## FORWARD LOOKING STATEMENTS

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

Although forward-looking statements in this report reflect the good faith judgment of our management, such statements can only be based on facts and factors currently known by us. Consequently, forward-looking statements are inherently subject to risks and uncertainties and actual results and outcomes may differ materially from the results and outcomes discussed in or anticipated by the forward-looking statements. Factors that could cause or contribute to such differences in results and outcomes include, without limitation, those specifically addressed under the headings “Risks Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our annual report on Form 10-K for the fiscal year ended December 31, 2014, as amended, and in this Form 10-Q and information contained in other reports that we file with the Securities and Exchange Commission (the “SEC”). You are urged not to place undue reliance on these forward-looking statements, which speak only as of the date of this report.

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

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

## PART I-FINANCIAL INFORMATION

## Item 1. Financial Statements

**RETROPHIN, INC. AND SUBSIDIARIES**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
(In thousands of U.S. dollars, except share and per share amounts)

	September 30, 2015	December 31, 2014
	(unaudited)	
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 140,596	\$ 18,204
Marketable securities	94,681	9,556
Accounts receivable, net	13,499	7,960
Inventory, net	2,650	801
Prepaid expenses and other current assets	2,002	813
Note receivable	46,526	—
Total current assets	299,954	37,334
Property and equipment, net	478	671
Other asset	2,006	2,265
Intangible assets, net	162,997	94,265
Goodwill	936	936
Long term note receivable	45,259	—
Total assets	\$ 511,630	\$ 135,471
<b>Liabilities and Stockholders' Equity (Deficit)</b>		
Current liabilities:		
Deferred technology purchase liability	\$ 1,000	\$ 1,000
Accounts payable	4,893	7,124
Accrued expenses	22,318	27,883
Other liability	1,027	938
Acquisition-related contingent consideration	5,069	2,118
Derivative financial instruments, warrants	42,120	27,990
Note payable	—	40,486
Short term deferred income tax liability, net	15,892	—
Taxes payable	9,628	—
Total current liabilities	101,947	107,539
Convertible debt	43,747	43,288
Other liability	13,915	12,234
Acquisition-related contingent consideration, less current portion	48,651	9,520
Long term deferred income tax liability, net	12,358	141
Total liabilities	220,618	172,722
<b>Stockholders' Equity (Deficit):</b>		
Preferred stock \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of September 30, 2015 and December 31, 2014	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 36,008,096 and 26,428,071 issued and 36,008,096 and 26,048,480 outstanding as of September 30, 2015 and December 31, 2014, respectively	4	3
Additional paid-in capital	353,794	140,851
Treasury stock, at cost, 0 and 379,591 shares as of September 30, 2015 and December 31, 2014, respectively	—	(3,215)
Accumulated deficit	(62,684)	(179,175)
Accumulated other comprehensive income (loss)	(102)	4,285
Total stockholders' equity (deficit)	291,012	(37,251)
Total liabilities and stockholders' equity (deficit)	\$ 511,630	\$ 135,471

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

**RETROPHIN, INC. AND SUBSIDIARIES**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND**  
**COMPREHENSIVE INCOME (LOSS)**

(In thousands of U.S. dollars, except share and per share amounts)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
		<i>(As Restated)</i>		<i>(As Restated)</i>
Net product sales	\$ 28,005	\$ 8,349	\$ 69,444	\$ 14,118
Operating expenses:				
Cost of goods sold	513	198	1,424	233
Research and development	14,064	12,646	34,974	32,899
Selling, general and administrative	22,308	17,372	56,856	42,097
Change in valuation of contingent consideration	6,906	—	7,026	—
Impairment of intangible assets	4,710	—	4,710	—
Total operating expenses	48,501	30,216	104,990	75,229
Operating loss	(20,496)	(21,867)	(35,546)	(61,111)
Other income (expenses), net:				
Litigation settlement gain	—	—	15,500	—
Other income (expense), net	(314)	170	35	545
Interest expense, net	(695)	(2,629)	(7,415)	(4,808)
Debt early payment penalty	—	—	(2,250)	—
Loss on extinguishment of debt	(4,151)	—	(4,151)	—
Finance expense	—	(13)	(600)	(4,721)
Change in fair value of derivative instruments	29,991	6,359	(36,180)	(14,276)
Gain on sale of assets	140,004	—	140,004	—
Bargain Purchase Gain	—	—	49,063	—
Total other income (expense), net	164,835	3,887	154,006	(23,260)
Income (Loss) before provision for income taxes	144,339	(17,980)	118,460	(84,371)
Income tax benefit (provision)	(38,761)	—	1,246	2,460
Net income (loss)	\$ 105,578	\$ (17,980)	\$ 119,706	\$ (81,911)
Net income (loss) per common share, basic	\$ 2.95	\$ (0.67)	\$ 3.67	\$ (3.25)
Net income (loss) per common share, diluted	\$ 1.78	\$ (0.84)	\$ 3.30	\$ (3.25)
Weighted average common shares outstanding, basic	35,741,877	26,682,510	32,650,408	25,229,847
Weighted average common shares outstanding, diluted	42,752,859	28,210,225	36,800,536	25,229,847
Comprehensive income (loss):				
Net income (loss)	\$ 105,578	\$ (17,980)	\$ 119,706	\$ (81,911)
Foreign currency translation	14	—	8	—
Unrealized gain (loss) on marketable securities	(876)	3,232	(4,395)	3,751
Comprehensive income (loss)	\$ 104,716	\$ (14,748)	\$ 115,319	\$ (78,160)

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

**RETROPHIN, INC. AND SUBSIDIARIES**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**  
(In thousands of U.S. dollars)  
(Unaudited)

	For the Nine Months Ended September 30,	
	2015	2014 <i>(As Restated)</i>
<b>Cash Flows From Operating Activities:</b>		
Net income (loss)	\$ 119,706	\$ (81,911)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Depreciation and amortization	9,417	3,543
Realized loss on marketable securities	293	(544)
Gain upon divestiture of Pediatric Priority Review Voucher	(140,004)	—
Gain upon divestiture of other assets	(200)	—
Deferred income tax provision	(11,791)	—
Amortization of debt discount and deferred financing costs	1,175	571
Lease liability	32	—
Settlement expense	—	5,684
Loss on early retirement of debt	4,151	—
Impairment of intangible assets	4,710	—
Loss on disposal of fixed assets	112	—
Bargain purchase gain	(49,063)	—
Share based compensation	18,748	9,237
Derivative financial instruments, warrants, issued, recorded in interest expense	1,050	—
Change in estimated fair value of derivative financial instruments, warrants	36,180	14,276
Change in fair value of contingent consideration	7,026	—
Non-cash financing cost	—	4,708
<b>Changes in operating assets and liabilities, net of acquisitions:</b>		
Accounts receivable	(5,540)	(4,676)
Inventory	(1,071)	(165)
Prepaid expenses and other assets	(1,712)	(1,242)
Accounts payable and accrued expenses	(852)	13,764
Income Taxes Payable	9,628	—
Net cash provided by (used in) operating activities	<u>1,995</u>	<u>(36,755)</u>
<b>Cash Flows From Investing Activities:</b>		
Purchase of fixed assets	(35)	(581)
Purchase of intangible assets	(5,271)	(3,302)
Proceeds from the sale of marketable securities	4,977	2,252
Purchase of marketable securities	(94,793)	(10,149)
Proceeds from securities sold, not yet purchased	—	7,500
Securities sold, not yet purchased	—	(5,310)
Cash paid for investment	—	(400)
Security deposits	—	(93)
Cash received upon sale of assets, net	148,411	—
Cash paid upon acquisition, net of cash acquired	(33,430)	(29,150)
Net cash provided by (used in) investing activities	<u>19,859</u>	<u>(39,233)</u>
<b>Cash Flows From Financing Activities:</b>		
Payment of acquisition-related contingent consideration	(2,498)	(562)
Payment of guaranteed minimum royalty	(527)	—
Proceeds from the exercise of warrants	4,372	8,337
Proceeds from the exercise of stock options	5,237	—
Purchase of treasury stock, at cost	—	(2,257)
Proceeds received from issuance of common stock	149,454	36,835
Financing costs from issuance of common stock	(9,500)	—
Proceeds from credit agreement	—	42,366
Proceeds from Note Purchase Agreement	—	42,924
Payment of other liability	(1,000)	(507)
Repayment of credit facility	(45,000)	—
Repayment of Manchester Note payable	—	(31,283)

Net cash provided by financing activities	100,538	95,853
Net increase in cash	122,392	19,865
Cash, beginning of year	18,204	5,997
Cash, end of period	\$ 140,596	\$ 25,862
Supplemental Disclosure of Cash Flow Information:		
Cash paid for interest	\$ 4,994	\$ 3,114
Non-cash investing and financing activities:		
Accrued royalty in excess of minimum payable to the sellers of Thiola®	\$ 2,237	\$ —
Reclassification of derivative liability to equity due to exercise of warrants	\$ 23,100	\$ 23,365
Present value of contingent consideration payable to sellers of Manchester Pharmaceuticals LLC	\$ —	\$ 12,238
Present value of guaranteed minimum royalty payable to sellers of Thiola®	\$ —	\$ 11,818
Note payable entered into upon consummation of Manchester Pharmaceuticals LLC	\$ —	\$ 31,283
Present value of contingent consideration payable to sellers of Asklepiion Pharmaceuticals LLC	\$ 42,010	\$ —
Shares issued in connection with Cholbam® acquisition	\$ 15,844	\$ —
Unrealized gain (loss) on marketable securities	\$ (4,395)	\$ 3,701
Unrealized loss on securities sold, not yet purchased	\$ —	\$ 34

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

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

**NOTE 1. DESCRIPTION OF BUSINESS***Organization and Description of Business*

In this Quarterly Report on Form 10-Q, 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. We are a fully integrated biopharmaceutical company with approximately 126 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<sup>®</sup> (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<sup>®</sup> 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.
- Thiola<sup>®</sup> (tiopronin) is approved in the United States for the prevention of cysteine (kidney) stone formation in patients with cystinuria.
- Cholbam<sup>®</sup> (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.

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 in vitro 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. We are currently enrolling patients for the DUET Phase 2 clinical study of sparsentan for the treatment of FSGS. Based 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. In the first quarter of 2015, sparsentan received orphan drug designation from the U.S. Food and Drug Administration (“FDA”).

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 ex-US 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 on May 5, 2015 and was granted fast track designation on June 4, 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.

*Voucher Sale*

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<sup>®</sup>.



On July 2, 2015, the Company sold and transferred the Pediatric PRV to Sanofi. Pursuant to the Company's agreement with Sanofi, the Company will receive \$245 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 asset value of the Pediatric PRV recorded in the financial statements as of June 30, 2015 was \$96.3 million. In accordance with accounting principles generally accepted in the United States ("GAAP"), the Company recorded the future short term and long term payments at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale. The incremental gain from the sale of the asset was approximately \$140.0 million during the three months ended September 30, 2015, net of \$4.9 million in fees contractually due as part of the Cholbam<sup>®</sup> acquisition.

On June 11, 2015, the General Court of the European Union annulled the marketing authorization ("MA") for Kolbam (bottled and branded name of Cholbam<sup>®</sup> in Europe) in recognition of the orphan market exclusivity afforded to Orphacol. This was the result of a lawsuit brought by a competitor product against the awarding of the MA for Kolbam by the EU Commission, citing similarities in the efficacy statements in a section of the labelling, and the competitor claimed circumvention of market exclusivity attached to Orphacol. The competitor product received its MA and orphan designation before Kolbam's MA, which purportedly entitled it to 10 years of market exclusivity. The EU Court ruled in favor of the competitor, and Kolbam's MA was withdrawn. The withdrawal of the MA was solely based on market exclusivity, and not for safety or efficacy reasons. At the time of the EU Court's ruling, the product was launched in one EU country, France.

Kolbam is currently available in Europe only through nationally approved Named Patient Programs.

On September 24, 2015, the European Committee for Medical Products for Human Use adopted an opinion recommending the Company be granted a MA for Kolbam. We anticipate a ruling from the EU Commission on the issuance of a new MA with revised labeling by the end of the year.

## **NOTE 2. BASIS OF PRESENTATION**

The accompanying unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements and notes thereto included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014 (the "2014 10-K") filed with the Securities and Exchange Commission (the "SEC") on March 11, 2015, and amended on March 13, 2015. The accompanying condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP") for interim financial information, the instructions to Form 10-Q and the rules and regulations of the SEC. Accordingly, since they are interim statements, the accompanying condensed consolidated financial statements do not include all of the information and notes required by GAAP for annual financial statements, but reflect all adjustments consisting of normal, recurring adjustments, that are necessary for a fair presentation of the financial position, results of operations and cash flows for the interim periods presented. Interim results are not necessarily indicative of the results that may be expected for any future periods. The December 31, 2014 balance sheet information was derived from the audited financial statements as of that date.

## **NOTE 3. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES**

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

### *Principles of Consolidation*

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

### *Inventory*

Inventories are stated at the lower of cost or market. We determine the cost of our inventories, which include amounts related to materials, third-party contract manufacturing and packaging services, on a first-in, first-out basis. We capitalize inventory costs at our suppliers when, based on management's judgment, the realization of future economic benefit is probable at each given supplier.

### *Restatement of Prior Quarters*

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"). Our Form 10-Q for the three and nine months ended September 30, 2014 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 at the Special Meeting of Stockholders on February 3, 2015. In addition,

our Form 10-Q for the three and nine months ended September 30, 2014 contained errors related to certain consulting agreements entered into by the Company, pursuant to which an expense and a settlement liability related to the entire amount of the stock to be issued under such consulting agreements should have been taken and revalued at each reporting period based on changes in the Company's stock price, until the stock had been entirely issued. We believe that the errors in the Form 10-Q for the three and nine months ended September 30, 2014 do not cause the financial statements included therein to be misleading, and therefore such financial statements can still be relied upon. However, we have corrected such errors, including any related disclosures, in this Form 10-Q. The impact for the three and nine months ended September 30, 2014, was a decrease to operating expenses of \$1.6 million, and an increase to operating expenses of \$0.2 million, respectively, and an decrease to net loss of \$1.6 million, and an increase to net loss of \$0.2 million, respectively.

#### *Recently Issued Accounting Pronouncements*

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 April 2015, the FASB issued ASU No. 2015-3, *Simplifying the Presentation of Debt Issuance Costs*. This standard amends existing guidance to require the presentation of debt issuance costs in the balance sheet as a deduction from the carrying amount of the related debt liability instead of a deferred charge. It is effective for annual reporting periods beginning after December 15, 2015, but early adoption is permitted. This accounting update will have an immaterial impact to the financial statements and the Company has chosen not to early adopt this standard.

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 September 2015, the FASB issued ASU No. 2015-16, *Simplifying the Accounting for Measurement-Period Adjustments*. This ASU eliminates the requirement for an acquirer in a business combination to account for measurement-period adjustments retrospectively. The ASU is effective for public business entities for fiscal years beginning after December 15, 2015, and interim periods within those fiscal years. Early adoption is permitted for any interim and annual financial statements that have not yet been issued. The ASU is applied prospectively to adjustments to provisional amounts that occur after the effective date. That is, the ASU applies to open measurement periods, regardless of the acquisition date. The Company is currently assessing the impact that adopting this new accounting guidance will have on its consolidated financial statements and footnotes disclosures.

#### **NOTE 4. INCOME TAXES**

The Company follows FASB 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. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled.

As required by the authoritative guidance on accounting for income taxes, we evaluate the realizability of deferred income tax assets on a jurisdictional basis at each reporting date. Accounting guidance for income taxes requires that a valuation allowance be established when it is more-likely-than-not that all or a portion of the deferred income tax assets will not be realized. Management assesses all available positive and negative evidence to determine whether or not the deferred income tax assets are more-likely-than-not realizable.

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 FASB 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. FASB ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. As of September 30, 2015 and December 31, 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 and has recorded an indemnification asset on the balance sheet.

In the first quarter of 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 income pursuant to FASB ASC 740, the Company was able to reduce its existing valuation allowance by \$39.9 million in the first quarter. The deferred tax liabilities supporting the ability to realize the deferred tax assets in the 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.

In the third quarter of 2015, the Company recorded total tax expense of \$38.8 million primarily relating to current and deferred tax expense accrued on the sale of Pediatric PRV, partially offset by the release of a valuation allowance pursuant to the utilization of net operating loss carry-forwards primarily related to the Pediatric PRV sale.

Our effective tax rate for the three months ended September 30, 2015 was 26.9% compared to 0% for the three months ended September 30, 2014. For the nine months ended September 30, 2015 and September 30, 2014, our effective tax rate was (1.1)% and 2.9%, respectively. Under GAAP, quarterly effective tax rates may vary significantly depending on the actual operating results in the various tax jurisdictions, significant transactions, as well as changes in the valuation allowance related to the expected recovery of deferred tax assets.

For the three months ended September 30, 2015, when compared to 2014, the increase in the effective income tax rate was primarily attributable to the current and deferred tax expense on the sale of the Pediatric PRV, partially offset by the release of a U.S. federal valuation allowance. For the nine months ended September 30, 2015, when compared to 2014, the decrease in the effective income tax rate was primarily attributable to the net benefit in 2015 resulting from valuation allowance released upon realization of substantially all of our Federal net operating losses, compared to tax expense during the first nine months of 2014 as a result of a decrease in the deferred tax liability associated with an indefinite lived asset.

At September 30, 2015, we had gross unrecognized tax benefits of \$0.4 million compared to \$0 at December 31, 2014, representing a net increase of \$0.4 million during the nine months ended September 30, 2015. Substantially all of the gross unrecognized tax benefits, if recognized, would impact our effective tax rate in the period of recognition. We did not recognize any interest and penalties related to unrecognized tax benefits.

We are currently not under income tax audit examination in any material jurisdictions in which we operate.

#### **NOTE 5. BUSINESS ACQUISITION**

##### *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 Asklepiion, all worldwide rights, titles, and ownership of Cholbam® (cholic acid) for the treatment of bile acid synthesis defects, if approved by the FDA. 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<sup>®</sup>, 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 may also be required to pay contingent consideration consisting of milestones and tiered royalties with a present value of \$39.1 million.

The 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 contemplate the Pediatric PRV because the issuance of a Pediatric PRV is extremely rare. Therefore when the FDA granted the Pediatric PRV with the Cholbam<sup>®</sup> approval, a 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<sup>®</sup>, 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 price allocation of \$91.3 million as of the acquisition completion date of March 31, 2015 was 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
<b>Fair Value of Assets Acquired and Liabilities Assumed</b>		
Acquired product rights-Cholbam <sup>®</sup> (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 completed in the first quarter is not presented, because the effects of such transaction is considered immaterial to the Company.

#### **NOTE 6. SHORT-TERM INVESTMENTS**

The Company's short-term investments as of September 30, 2015 are 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.

Investments consist of the following (*in thousands*):

	September 30, 2015	December 31, 2014
<b>Marketable Equity Securities:</b>		
Common Stock	\$ —	\$ 9,556
<b>Marketable Other than Equity Securities:</b>		
Commercial paper	11,930	—
Corporate debt securities	72,746	—
Securities of government sponsored entities	10,005	—
<b>Total Marketable Securities:</b>	<b>\$ 94,681</b>	<b>\$ 9,556</b>

The following is a summary of short-term marketable securities classified as available-for-sale as of September 30, 2015 (*in thousands*):

	Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
<b>Marketable Other than Equity Securities:</b>					
Commercial paper	Less than 1	\$ 11,935	\$ —	\$ (5)	\$ 11,930
Corporate debt securities	Less than 1	34,123	1	(42)	34,082
Securities of government-sponsored entities	Less than 1	5,003	—	—	5,003
Total maturity less than 1 year		51,061	1	(47)	51,015
Corporate debt securities	1 to 2	38,732	17	(85)	38,664
Securities of government-sponsored entities	1 to 2	5,000	2	—	5,002
Total maturity 1 to 2 years		43,732	19	(85)	43,666
Total available-for-sale securities		<u>\$ 94,793</u>	<u>\$ 20</u>	<u>\$ (132)</u>	<u>\$ 94,681</u>

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
<b>Marketable Equity Securities:</b>				
Common Stock	\$ 5,160	\$ 4,499	\$ (103)	\$ 9,556
Total available-for-sale securities	<u>\$ 5,160</u>	<u>\$ 4,499</u>	<u>\$ (103)</u>	<u>\$ 9,556</u>

The following table presents information about available-for-sale investments in an unrealized loss position as of September 30, 2015 (*in thousands*):

	Less Than 12 Months		12 Months or Greater		Total	
	Estimated Fair Value	Unrealized Losses	Estimated Fair Value	Unrealized Losses	Estimated Fair Value	Unrealized Losses
<b>September 30, 2015</b>						
Commercial paper	\$ 11,930	\$ (5)	\$ —	\$ —	\$ 11,930	\$ (5)
Corporate debt securities	62,492	(127)	—	—	62,492	(127)
Securities of government-sponsored entities	3,002	—	—	—	3,002	—
<b>Total</b>	<u>\$ 77,424</u>	<u>\$ (132)</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 77,424</u>	<u>\$ (132)</u>

The primary objective of the Company's investment portfolio is to enhance overall returns while preserving capital and liquidity. The Company's investment policy limits interest-bearing security investments to certain types of instruments issued

by institutions with primarily investment grade credit ratings and places restrictions on maturities and concentration by asset class and issuer.

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 September 30, 2015 and December 31, 2014, the Company believed the cost basis for available-for-sale investments were recoverable in all material respects.

#### **NOTE 7. DERIVATIVE FINANCIAL INSTRUMENTS**

The Company accounts for derivative financial instruments in accordance with FASB ASC 815-40, *Derivative and Hedging – Contracts in Entity's Own Equity* ("ASC 815-40"), pursuant to which 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.

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 adjustments within other income (expenses), net, in the Company's accompanying condensed consolidated statements of operations and comprehensive income (loss). The Company recorded a gain on the change in the estimated fair value of warrants of \$30.0 million and \$6.4 million for the three months ended September 30, 2015 and 2014, respectively, and a loss on the change in the estimated fair value of warrants of \$36.2 million and \$14.3 for the nine months ended September 30, 2015 and 2014, respectively.

The Company calculated the fair value of the warrants using the Monte Carlo Simulation as of September 30, 2015 and the Binomial Lattice options pricing model as of December 31, 2014, using the following assumptions:

	September 30, 2015	December 31, 2014
Fair value of common stock	\$ 20.26	\$ 12.24
Expected life (in years), represents the weighted average period until next liquidity event	n/a**	0.33
Remaining life of the warrants (in years)	2.4 - 4.3	3.1 - 4.9
Risk-free interest rate	.75% – 1.21%	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.

#### **NOTE 8. FAIR VALUE MEASUREMENTS**

##### *Financial Instruments and Fair Value*

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

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

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

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

The valuation techniques used to measure the fair value of the Company’s debt instruments and all other financial instruments, all of which have counterparties with high credit ratings, were valued based on quoted market prices or model driven valuations using significant inputs derived from or corroborated by observable market data.

In estimating the fair value of the Company’s derivative liabilities, the Company used the Monte Carlo Simulation as of September 30, 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, accounts receivable, deposits on license agreements, accounts payable and the credit facility, due to their short term nature. In estimating the fair value of convertible debt which approximates the carrying value, we considered factors such as market conditions, prepayment and make-whole provisions, variability in pricing from multiple lenders and term of debt.

The following table presents the Company’s assets 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 September 30, 2015 (*in thousands*):

	As of September 30, 2015	Fair Value Hierarchy at September 30, 2015		
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
<b>Asset:</b>				
Marketable securities, available-for-sale	\$ 94,681	\$ —	\$ 94,681	\$ —
<b>Liabilities:</b>				
Derivative liability related to warrants	\$ 42,120	\$ —	\$ —	\$ 42,120
Contingent consideration	\$ 53,720	\$ —	\$ —	\$ 53,720

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*):

	As of December 31, 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)	Significant unobservable inputs (Level 3)
<b>Asset:</b>				
Marketable securities, available-for-sale	\$ 9,556	\$ 9,556	\$ —	\$ —
<b>Liabilities:</b>				
Derivative liability related to warrants	\$ 27,990	\$ —	\$ —	\$ 27,990
Acquisition-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 derivative financial instruments, warrants liability for the nine months ended September 30, 2015 (*in thousands*):

	<b>Fair Value Measurements of Common Stock Warrants Using Significant Unobservable Inputs (Level 3)</b>
Balance at January 1, 2015	\$ 27,990
Reclassification of derivative liability to equity upon exercise of warrants	(23,100)
Issuance of Warrants	1,050
Change in estimated fair value of liability classified warrants	36,180
Balance at September 30, 2015	<u>\$ 42,120</u>

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 acquisition-related contingent consideration for the nine months ended September 30, 2015 (*in thousands*):

	<b>Fair Value Measurements of Acquisition-Related Contingent Consideration (Level 3)</b>
Balance at January 1, 2015	\$ 11,637
Present value of contingent consideration of Cholbam <sup>®</sup> , upon acquisition	39,107
Increase from revaluation of contingent consideration	7,026
Decrease of contingent consideration, asset divestiture	(604)
Contractual Payments	(2,498)
Contractual Payments Accrued at September 30, 2015	(948)
Balance at September 30, 2015	<u>\$ 53,720</u>

The fair value of contingent consideration liabilities was determined by applying a form of the income approach (a level 3 input), based upon the probability-weighted projected payment amounts discounted to present value at a rate appropriate for the risk of achieving the performance targets. The key assumptions included in the calculations were the earn-out period payment probabilities, projected revenues, discount rate and the timing of payments. The present value of the expected payments considers the time at which the obligations are expected to be settled and a discount rate that reflects the risk associated with the performance payments.

During the nine month period ended September 30, 2015, the Company incurred a charge of \$7.0 million in operating expenses on the condensed consolidated statement of operations and comprehensive income (loss), \$6.6 million of which is related to the increase in contingent consideration liabilities for the product Chenodal<sup>®</sup>. The primary drivers for the change were projected revenues and timing of payments.

On September 30, 2015, the Company wrote-off the entire value of intangible assets related to Carbetocin (a level 3 input). 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 is included in operating expenses on the Company's Condensed Consolidated Statement of Operations and Comprehensive Income (Loss).

#### **NOTE 9. INTANGIBLE ASSETS**

As of September 30, 2015, the net book value of amortizable intangible assets was approximately \$163.0 million.

Amortizable intangible assets as of September 30, 2015 and December 31, 2014 (*in thousands*):



	September 30, 2015	December 31, 2014
Finite-lived intangibles Assets	\$ 176,749	\$ 99,867
Less: Accumulated amortization	(13,752)	(5,602)
Net carrying value	<u>\$ 162,997</u>	<u>\$ 94,265</u>

The following table summarizes amortization expense for the three and nine months ended September 30, 2015 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Research and development	\$ 200	\$ 261	\$ 614	\$ 566
Selling, general and administrative	3,675	1,554	8,803	2,901
Total amortization expense	<u>\$ 3,875</u>	<u>\$ 1,815</u>	<u>\$ 9,417</u>	<u>\$ 3,467</u>

On September 30, 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 condensed consolidated statement of operations and comprehensive income (loss).

#### **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 \$46.0 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. The Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with the terms. At September 30, 2015 and December 31, 2014, the aggregate carrying value of the Notes was \$43.7 million and \$43.3, respectively, which bore a weighted average annual interest rate of 4.5% during the nine months ended September 30, 2015 and December 31, 2014, respectively.

##### *Credit Facility*

On June 30, 2014, the Company entered into a \$45 million Credit Agreement ("Credit Facility") which was schedule 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.

At December 31, 2014, the Company reclassified the note payable to short term due to its inability to meet debt covenant requirements related to its cash balances over the next twelve months. On March 24, 2015, the Company received \$140.0 million in proceeds from its equity financing, therefore, the Company expected to have the ability to meet future covenant requirements and reclassified the debt to long term.

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 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 \$30.0 million, which could be 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 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 condensed consolidated statement of operations and comprehensive income (loss), for the write-off of the debt discount and issuances for the Credit Facility.

At September 30, 2015 and December 31, 2014, the aggregate carrying value of the Credit Facility was \$0.0 million and \$40.5 million, respectively, which bore a weighted average annual interest rate of 11.0% during the periods outstanding.

Total interest expense, net, recognized was \$0.7 million and \$2.6 million for the three months ended September 30, 2015 and 2014, respectively, and \$7.4 million and \$4.8 million for the nine months ended September 30, 2015 and 2014, respectively.

#### *Debt Maturities*

The stated maturities of the Company's long-term debt are as follows as of September 30, 2015 (*in thousands*):

2015	\$	—
2016		—
2017		—
2018		—
2019		46,000
Thereafter		—
	<u>\$</u>	<u>46,000</u>

#### **NOTE 11. ACCRUED EXPENSES**

Accrued expenses at September 30, 2015 and December 31, 2014 consisted of the following (*in thousands*):

	September 30, 2015	December 31, 2014
Government rebates payable	\$ 5,226	\$ 1,353
Compensation related costs	5,094	8,163
Accrued royalties	4,249	—
Research and development	2,222	3,720
Selling, general and administrative	1,807	2,411
Severance related costs	1,548	5,710
Accounting and legal fees	1,186	1,208
Interest	799	2,318
Miscellaneous accrued	187	—
License fee	—	3,000
	<u>\$ 22,318</u>	<u>\$ 27,883</u>

#### **NOTE 12. INCOME (LOSS) PER SHARE**

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

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

	Three Months Ended					
	September 30, 2015			September 30, 2014		
	Shares	Net Income	EPS	Shares	Net Income	EPS
Basic Earnings per Share	35,741,877	\$ 105,578	\$ 2.95	26,682,510	\$ (17,980)	\$ (0.67)
Dilutive shares related to warrants	—	(29,991)	—	—	(5,689)	—
Change in fair value of derivative instruments	2,053,934	—	—	1,527,715	—	—
Convertible Debt	2,642,160	518	—	—	—	—
Restricted Stock	409,166	—	—	—	—	—
Stock Options	1,905,722	—	—	—	—	—
Dilutive Earnings per Share	42,752,859	\$ 76,105	\$ 1.78	28,210,225	\$ (23,669)	\$ (0.84)

	Nine Months Ended					
	September 30, 2015			September 30, 2014		
	Shares	Net Income	EPS	Shares	Net Income	EPS
Basic Earnings per Share	32,650,408	\$ 119,706	\$ 3.67	25,229,847	\$ (81,911)	\$ (3.25)
Convertible Debt	2,642,160	1,553	—	—	—	—
Restricted Stock	331,073	—	—	—	—	—
Stock Options	1,176,895	—	—	—	—	—
Dilutive Earnings per Share	36,800,536	\$ 121,259	\$ 3.30	25,229,847	\$ (81,911)	\$ (3.25)

For the three and nine months ended September 30, 2015 and 2014, the following shares were excluded because they were anti-dilutive:

	Three Months Ended		Nine Months Ended	
	September 30, 2015	September 30, 2014	September 30, 2015	September 30, 2014
	Restricted Stock	26,033	438,416	41,132
Convertible Debt	—	2,642,160	—	3,106,345
Options	1,762,891	2,852,500	1,083,093	2,852,500
Warrants	—	337,500	2,691,589	337,500
Total Anti-Dilutive Shares	1,788,924	6,270,576	3,815,814	6,763,507

### NOTE 13. COMMITMENTS AND CONTINGENCIES

#### Leases and Sublease Agreements

On October 1, 2013, the Company entered into building lease for office space located at One Kendall Square in Cambridge, Massachusetts. In August 2014, Retrophin ceased use of the facility at One Kendall Square and all employees formerly located at this facility moved into the new facility on Binney Street, Cambridge, Massachusetts. In March 2015, the Company entered into a termination agreement with the landlord and paid an \$80,000 lease termination fee.

On February 28, 2014, the Company amended its lease agreement for its offices located in Carlsbad, California. In October 2014, Retrophin ceased use of this facility, and all employees formerly located at that facility moved into the new headquarters facility in San Diego, California. In March 2015, the Company entered into an agreement to sublease a portion of the Carlsbad California lease for a three year term with an annual rent of approximately \$56,000, with annual rent escalations.

#### Research Collaboration and Licensing Agreements

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

the Company to pay royalties, in the event the Company sells or licenses any proprietary products developed under the respective agreements.

### Contractual Commitments

The following table summarizes our principal contractual commitments, excluding open orders that support normal operations, as of September 30, 2015 (*in thousands*):

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating Leases	\$ 1,851	\$ 797	\$ 1,054	\$ —	\$ —
Other commitments	4,524	1,029	1,133	870	1,492
	\$ 6,375	\$ 1,826	\$ 2,187	\$ 870	\$ 1,492

### Legal Proceedings

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.

On September 19, 2014, a purported shareholder 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 plaintiff seeks, 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 parties have reached an agreement to settle the lawsuit. On October 19, 2015, Plaintiffs filed a motion seeking the Court’s approval of the settlement. If the Court approves the settlement, Mr. Shkreli will pay \$2,025,000 to the Company and will pay an additional \$625,000 to Plaintiffs to compensate them for their legal fees.

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 on June 26, 2015. Plaintiffs filed a consolidated opposition to the motions on July 27, 2015. Defendants filed their reply briefs in support of the motions on October 28, 2015. 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. The Company has also been cooperating with a parallel investigation by the U.S. Securities and Exchange Commission.

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 are currently selecting 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 September 30, 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.

#### **NOTE 14. STOCKHOLDERS' EQUITY/DEFICIT**

##### *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.

##### *Restricted Shares*

The following table summarizes the Company's restricted stock activity during the nine months ended September 30, 2015:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Outstanding December 31, 2014	691,668	\$ 10.83
Granted	253,000	
Vested	(159,666)	
Forfeited/canceled	(66,668)	
Outstanding September 30, 2015	718,334	\$ 16.89

##### *Stock Options*

The following table summarizes information about stock option activity during the nine months ended September 30, 2015:

	Shares Underlying Options	Weighted Average Exercise Price
Outstanding at December 31, 2014	4,892,208	\$ 10.93
Granted	2,095,000	
Exercised	(401,740)	
Forfeited/canceled	(842,875)	
Outstanding at September 30, 2015	5,742,593	\$ 16.86

At September 30, 2015, outstanding options to purchase 1.8 million shares were exercisable with a weighted-average exercise price per share of \$11.69.

##### *Share Based Compensation*

Total non-cash stock-based compensation by operating statement classification is as follows for the three and nine months ended September 30, 2015 and 2014 (*in thousands*):

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Research and Development	\$ 2,692	\$ 1,039	\$ 6,660	\$ 1,997
Selling, General & Administrative	5,489	2,075	12,088	4,877
Selling, General & Administrative - Consultants	—	90	—	2,363
Total	\$ 8,181	\$ 3,204	\$ 18,748	\$ 9,237

### Exercise of Warrants

During the three and nine months ended September 30, 2015, the Company issued 630,557 and 818,610 shares of common stock upon the exercise of warrants, respectively, pursuant to which the Company received \$3.8 million and \$4.3 million, respectively. The Company reclassified \$19.1 million and \$23.1 million of the derivative liability as equity for the value of these warrants on the date of exercise for the three and nine months ended September 30, 2015, respectively. 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 condensed consolidated financial statements of the Company. The number of warrants outstanding at September 30, 2015 was 2,691,589, and the number of warrants outstanding at December 31, 2014 was 3,421,255.

### NOTE 15. SALE OF ASSETS

On January 9, 2015, the Company entered into an asset purchase agreement with Turing Pharmaceuticals A.G. (“Turing Pharmaceuticals”), pursuant to which the Company sold Turing Pharmaceuticals its ketamine licenses and assets (the “Assets”) for a purchase price of \$1.0 million. Turing Pharmaceuticals also assumed all future liabilities related to the Assets.

On February 12, 2015, the Company, its wholly-owned subsidiaries Manchester Pharmaceuticals LLC (“Manchester”) and Retrophin Therapeutics International, LLC (collectively, the “Sellers”), entered into a purchase agreement with Waldun Pharmaceuticals, LLC (“Waldun”), pursuant to which the Sellers sold Waldun their product rights to Vecamyl for a purchase price of \$0.7 million. Waldun in turn sold Vecamyl to Turing Pharmaceuticals. In connection therewith, on February 12, 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 also assumed certain liabilities related to the Vecamyl product rights and inventory.

On February 12, 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 assumed certain liabilities related to the Syntocinon assets and licenses.

In conjunction with the sale of the Vecamyl, Syntocinon and ketamine assets, the Company recorded a gain of \$0.2 million and wrote off the unamortized book value of the Vecamyl Product Rights Intangible Asset of \$3.3 million and Syntocinon License Intangible Asset of \$4.8 million.

On July 2, 2015, the Company completed the sale and transfer of the Pediatric PRV to Sanofi. Pursuant to the Company’s agreement with Sanofi, the Company will receive \$245 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 asset value of the Pediatric PRV was recorded at \$96.3 million. In accordance with GAAP the Company recorded the future short term and long term payments at present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale. The incremental gain from the sale of the asset was \$140.0 million recorded during the 3 months ended September 30, 2015, net of \$4.9 million in fees contractually due as part of the Cholbam<sup>®</sup> acquisition.

As of September 30, 2015, the present value of the two future payments from Sanofi related to the Pediatric PRV sale were \$46.5 million, short term, and \$45.3 million, long term. Imputed interest of \$0.6 million was recorded during the period to interest income.

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

*The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in this Quarterly Report on Form 10-Q and the audited financial statements and notes thereto as of and for the year ended December 31, 2014 and the related Management’s Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our Annual Report on*

Form 10-K for the year ended December 31, 2014, filed with the Securities and Exchange Commission (SEC) on March 11, 2015, and amended on March 13, 2015. Past operating results are not necessarily indicative of results that may occur in future periods.

### **Forward-Looking Statements**

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

### **Overview**

We are a fully integrated biopharmaceutical company with approximately 126 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<sup>®</sup> (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<sup>®</sup> 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.
- Thiola<sup>®</sup> (tiopronin) is approved in the United States for the prevention of cysteine (kidney) stone formation in patients with severe homozygous cystinuria.
- Cholbam<sup>®</sup> (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.

On January 12, 2015, the Company announced the signing of a definitive agreement under which it acquired the exclusive right to purchase from Asklepios Pharmaceuticals, LLC (“Asklepios”), all worldwide rights, titles, and ownership of Cholbam<sup>®</sup> (cholic acid) for the treatment of bile acid synthesis defects, if approved by the FDA. Under the terms of the agreement, Retrophin paid Asklepios 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<sup>®</sup>.

The FDA granted Asklepios 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 original terms of the agreement with Asklepios to purchase Cholbam<sup>®</sup>.

On July 2, 2015, the Company sold and transferred the Pediatric PRV to Sanofi for \$245 million; at closing the Company received \$150.0 million, and \$47.5 million is due on the first and second anniversaries of the closing. The Company recorded the receivables at present value present on the balance sheet. The asset value of the Pediatric PRV was recognized at \$96.3 million, and therefore an incremental gain of approximately \$140.0 million, net, was recorded as of September 30, 2015, resulting from the sale of the Pediatric PRV.

On November 4, 2015 the Company received a letter from the United States Senate Special Committee on Aging requesting information on the pricing, distribution, and other financial information relating to the licensed product Thiola<sup>®</sup>. The Company is reviewing the letter and intends to cooperate with the request.



**Products and Research and Development Programs**

Product/Program	Pre-Clinical	Phase I	Phase II	Phase III	Market
<b>Cholbam:</b>					
Bile acid synthesis disorders due to single enzyme defects					
Peroxisomal disorders					
<b>Thiola:</b>					
Cystinuria					
<b>Chenodal:</b>					
Gallstones/CTX*					
<b>Sparsentan:</b>					
Focal Segmental Glomerulosclerosis					
<b>RE-024:</b>					
Pantothenate Kinase Associated Neurodegeneration					
<b>RE-034:</b>					
Multiple Indications					
<b>Discovery Programs:</b>					
Undisclosed Rare Disease Targets					

\* Chenodal is not currently indicated for CTX but is the standard of care for that disease.

*Cholbam®*

On March 18, 2015, the Company announced that the U.S. Food and Drug Administration (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 as well. 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 cysteine transport disorder that causes high cysteine levels in the urine and the formation of recurring kidney stones. The resulting long-term damage can cause long term kidney damage in addition to substantial pain and loss of productivity associated with renal colic and stone passage. The Company believes there are 10,000-12,000 patients with cystinuria in the United States, of which 4,000-5,000 patients may be appropriate candidates for treatment. We have built a salesforce to promote Thiola® to targeted physicians and are exploring alternative formulations that may enhance the value of Thiola® for patients with cystinuria.

*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. Discussions with the FDA have been initiated to determine the path for revising the Chenodal® label to include CTX, a rare genetic disease which causes multiple symptoms including neurologic impairment. Chenodeoxycholic acid has been utilized as the standard of care for CTX patients for several decades.



### 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 in vitro selectivity toward endothelin receptor type A. 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 expect approximately 100 patients to be enrolled. 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. In the first quarter of 2015, sparsentan was granted orphan drug designation.

### RE-024

The Company is 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 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 ex-US 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 on May 5, 2015 and was granted fast track designation on June 4, 2015.

### RE-034

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 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 process that allows modulation of the release of the active ingredient from the site of administration. The Company continues preclinical development of RE-034 to enable multiple strategic options.

## Results of Operations

### Results of Operations for the three and nine month periods ended September 30, 2015 compared to the three and nine month periods ended September 30, 2014.

#### Net Product Sales:

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Increase	2015	2014	Increase
Net product sales	\$ 28,005	\$ 8,349	\$ 19,656	\$ 69,444	\$ 14,118	\$ 55,326

The increase in sales for the three months ended September 30, 2015 over the prior year is due to increased patient counts for Chenodal<sup>®</sup> and Thiola<sup>®</sup>, and our acquisition of Cholbam<sup>®</sup> on March 31, 2015.

The increase in sales for the nine months ended September 30, 2015 over the prior year is due to our first sales being generated in March of 2014 after completing the acquisition of all of the membership interests of Manchester Pharmaceuticals, LLC (“Manchester”) for Chenodal<sup>®</sup> and Vecamyl<sup>®</sup>, entering into a license agreement in May 2014 with Mission Pharmacal for the U.S. marketing rights to Thiola<sup>®</sup>, and acquiring Cholbam<sup>®</sup> on March 31, 2015.

*Operating Expenses:*

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Increase	2015	2014	Increase
Cost of goods sold	\$ 513	\$ 198	\$ 315	\$ 1,424	\$ 233	\$ 1,191
Research and development	14,064	12,646	1,418	34,974	32,899	2,075
Selling, general and administrative	22,308	17,372	4,936	56,856	42,097	14,759
Change in valuation of contingent consideration	6,906	—	6,906	7,026	—	7,026
Impairment of intangible assets	4,710	—	4,710	4,710	—	4,710
	<u>\$ 48,501</u>	<u>\$ 30,216</u>	<u>\$ 18,285</u>	<u>\$ 104,990</u>	<u>\$ 75,229</u>	<u>\$ 29,761</u>

Selling, general and administrative expenses primarily include salary and benefit costs for employees included in our sales, marketing, finance, legal and administrative organizations, outside legal and professional services, amortization and facilities costs. The increase in selling, general and administrative expenses of \$4.9 million and \$14.8 million for the three and nine months ended September 30, 2015 as compared to 2014, respectively, is primarily due to an increase in personnel, including sales and marketing personnel, to support our commercial marketing in the current year and amortization from intangible assets.

We make significant investments in research and development in support of our development programs. Research and development costs are expensed as incurred and primarily include salary and benefit costs, fees paid to clinical research organizations, and supply costs. The increase in research and development costs of \$1.4 million for the three months ended September 30, 2015 as compared to 2014 is primarily due to increased compensation and clinical trial expense in support of the Company's lead development candidate, sparsentan.

The increase in research and development costs of \$2.1 million for the nine months ended September 30, 2015 as compared to 2014 is primarily due to increased headcount and compensation related to the hiring of critical regulatory and development expertise. This was partially offset by the timing of RE-024 toxicology studies which occurred during 2014.

During the three and nine month period ended September 30, 2015, the Company incurred a charge of \$6.9 and \$7.0 million in operating expenses on the condensed consolidated statement of operations and comprehensive income (loss), \$6.6 million of which is related to the increase in contingent consideration liabilities for the product Chenodal®. The primary driver for the change was projected revenues.

On September 30, 2015, the Company wrote-off \$4.7 million of intangible assets related to Carbetocin as the Company elected not to pursue any internal development of the asset.

*Other Income/Expenses:*

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Variance	2015	2014	Variance
Litigation settlement	\$ —	\$ —	\$ —	\$ 15,500	\$ —	\$ 15,500
Other income (expense), net	(314)	170	(484)	35	545	(510)
Interest expense, net	(695)	(2,629)	1,934	(7,415)	(4,808)	(2,607)
Early payment penalty	—	—	—	(2,250)	—	(2,250)
Loss on extinguishment of debt	(4,151)	—	(4,151)	(4,151)	—	(4,151)
Finance expense	—	(13)	13	(600)	(4,721)	4,121
Change in fair value of derivative instruments-gain (loss)	29,991	6,359	23,632	(36,180)	(14,276)	(21,904)
Gain on disposal of assets	140,004	—	140,004	140,004	—	140,004
Bargain purchase gain	—	—	—	49,063	—	49,063
	<u>\$ 164,835</u>	<u>\$ 3,887</u>		<u>\$ 154,006</u>	<u>\$ (23,260)</u>	

For the three months ended September 30, 2015, other income/expense variance was primarily attributable to the gain on the sale of the Pediatric PRV to Sanofi and the change in fair value of derivative instruments, driven by the decrease in the Company's stock price.

For the nine months ended September 30, 2015, other income/expense variance was primarily due to the gain on the sale of the Pediatric PRV to Sanofi, the bargain purchase gain on the Cholbam<sup>®</sup> acquisition net of tax, offset by the change in fair value of derivative instruments, driven by the increase in the Company's stock price.

*Income Tax Benefit (Provision):* In the third quarter of 2015, the Company recorded total tax expense of \$38.8 million primarily relating to current and deferred tax expense accrued on the sale of Pediatric PRV, partially offset by the release of a valuation allowance pursuant to the utilization of net operating loss carry-forwards primarily related to the Pediatric PRV sale.

**Liquidity and Capital Resources**

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 declared effective by the SEC on March 13, 2015. The shelf registration statement allowed us to issue shares of our common stock, preferred stock, debt securities and warrants, up to a total aggregate offering price of \$125.0 million from time to time in one or more offerings. As of March 24, 2015, we had sold all amounts available under this shelf registration statement.

On July 2, 2015, the Company sold and transferred the Pediatric PRV to Sanofi. Pursuant to the Company's agreement with Sanofi, the Company will receive \$245 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 asset value of the Pediatric PRV recorded in the financial statements as of June 30, 2015 was \$96.3 million. In accordance with GAAP the Company recorded the future payments at present value of \$46.2 million and \$44.9 million, respectively at the date of the sale. The incremental gain from the sale of the asset was \$140.0 million recorded as of September 30, 2015, net of \$4.9 million of fees contractually due as part of the Cholbam<sup>®</sup> acquisition.

We believe that our existing capital resources and projected revenues will be sufficient to satisfy our current and projected funding requirements for at least the next 12 months. However, we cannot guarantee that these capital resources and projected revenues will be sufficient to maintain all of our research or development programs and commercialization operations. The amount and timing of expenditures will vary depending upon a number of factors.

We cannot assure you that adequate funding will be available on terms acceptable to us, if at all. Any additional equity financings will be dilutive to our stockholders and any additional debt may involve operating covenants that may restrict our business. If adequate funds are not available through these means, we may be required to curtail our research or development programs and

commercialization operations. We cannot assure you that we will successfully generate revenues sufficient to enable us to earn a profit.

#### *Convertible Notes Payable*

On May 29, 2014, the Company entered into a Note Purchase Agreement relating to a private placement by the Company of \$46.0 million aggregate principal senior convertible notes due 2019 (the "Notes") which are convertible into shares of the Company's common stock at an initial conversion price of \$17.41 per share. The conversion price is subject to customary anti-dilution protection. The Notes bear interest at a rate of 4.5% per annum, payable semiannually in arrears on May 15 and November 15 of each year. The Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with the terms. On September 30, 2015 and December 31, 2014, the aggregate carrying value of the Notes was \$43.7 million and \$43.3, respectively, which bore a weighted average annual interest rate of 4.5% during the nine months ended September 30, 2015 and December 31, 2014, respectively.

#### *Credit Facility*

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.

#### *Cash Flows from Operating Activities*

Cash provided by operating activities was \$2.0 million for the nine month period ended September 30, 2015 compared to \$36.8 million used in the nine month period ended September 30, 2014. The increase of \$38.8 million was the result of a \$55.3 million increase in revenue partially offset by a \$29.8 million increase in operating expenses.

For the three months ended September 30, 2015, the Company generated positive cash flow from operating activities. Cash provided from operations of \$1.4 million was predominately due to revenue growth. The Company may fluctuate between cash used and cash provided by operations in the coming quarters depending upon levels of product revenue, commercialization expenses, other SG&A expenses and Research and Development expenditures to further its pipeline assets.

#### *Cash Flows from Investing Activities*

Cash provided by investing activities for the nine month period ended September 30, 2015 was \$19.9 million compared to \$39.2 million used for the nine month period ended September 30, 2014. The increase of \$59.1 million was the result of \$148.4 million in net cash received for the divestiture of assets in 2015, offset by lower cash used to purchase marketable securities in the prior year.

#### *Cash Flows from Financing Activities*

For the nine month period ended September 30, 2015, cash provided by financing activities was \$100.5 million compared to \$95.9 million during the nine month period ended September 30, 2014. In 2015, the cash was provided from the Company's follow-on public offering of \$140 million, net of fees, offset by the \$45 million repayment of the outstanding credit facility. In 2014, cash was primarily provided by the Company's \$36.8 million follow-on public offering and the proceeds of \$85.3 million from the issuance of debt, offset by the repayment of the Manchester Note payable of \$31.3 million.

### **Other Matters**

#### *Recently Issued Accounting Pronouncements*

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers*. While the standard supersedes existing revenue recognition guidance, it closely aligns with current GAAP. Under the new standard, revenue is recognized at the time a good or service is transferred to a customer for the amount 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 April 1, 2015, the FASB proposed deferring the effective date by one year to December 15, 2017 for annual reporting periods beginning after that date. The FASB also proposed permitting early adoption of the standard, but not before the original effective date of December

15, 2016. We are currently evaluating the impact, if any, the adoption of this standard will have on our consolidated financial statements.

In April 2015, the FASB issued ASU No. 2015-03, *Simplifying the Presentation of Debt Issuance Costs*. This standard amends existing guidance to require the presentation of debt issuance costs in the balance sheet as a deduction from the carrying amount of the related debt liability instead of a deferred charge. It is effective for annual reporting periods beginning after December 15, 2015, but early adoption is permitted. This accounting update has an immaterial impact to the financial statements and the Company has chosen not to early adopt this standard.

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 amendment is 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.

#### *Emerging Growth Company Critical Accounting Policy Disclosure*

We qualify as an “emerging growth company” under the Jumpstart Our Business Startups Action of 2012 (“JOBS Act”). Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. As an emerging growth company, we can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to take advantage of the benefits of this extended transition period.

#### **Off Balance Sheet Transactions**

None.

#### **Item 3. Quantitative and Qualitative Disclosures about Market Risk**

We invest our excess cash primarily in United States Government backed securities, asset-backed securities, and debt instruments of financial institutions and corporations with investment-grade credit ratings. These instruments have various short-term maturities. We do not utilize derivative financial instruments, derivative commodity instruments, or other market risk sensitive instruments, positions or transactions in any material fashion. Accordingly, we believe that, while the instruments held are subject to changes in the financial standing of the issuer of such securities, we are not subject to any material risks arising from changes in interest rates, foreign currency exchange rates, commodity prices, equity prices or other market changes that affect market risk sensitive investments. A hypothetical 1% adverse move in interest rates along the entire interest rate yield curve would not materially affect the fair value of our financial instruments that are exposed to changes in interest rates.

#### **Item 4. Controls and Procedures**

##### *Evaluation of Disclosure Controls and Procedures*

As of September 30, 2015, an evaluation was performed under the supervision and with the participation of our management, including our Chief Executive Officer (referred to as our CEO) and our Chief Financial Officer (referred to as our CFO), of the effectiveness of the design and operation of our disclosure controls and procedures. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management necessarily is required to apply its judgement in evaluating the cost-benefit relationship of possible controls and procedures. Based on that evaluation, our management, including our CEO and CFO, concluded that our disclosure controls and procedures were not effective at a reasonable level of assurance as of September 30, 2015, however, we are in the process of documenting and testing our internal control over financial reporting in order to report on the effectiveness of our internal controls as of December 31, 2015.

Our management does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all error and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, or misstatement, due

to error, if any, with the company have been detected. While we believe that our disclosure controls and procedures and internal control over financial reporting are and have been effective, in light of the foregoing we intend to continue to examine and refine our disclosure controls and procedures and internal control over financial reporting.

An evaluation was also performed under the supervision and with the participation of our management, including our CEO and CFO, of any changes in our internal control over financial reporting that occurred during our last fiscal quarter and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. That evaluation did not identify any change in our internal control over financial reporting that occurred during our latest fiscal quarter and that has materially affected, or is reasonably likely to affect, our internal control over financial reporting, except those controls that were added to remediate the material weaknesses disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2014 (the 2014 Form 10-K), and also disclosed below.

As of December 31, 2014, we carried out an assessment of the effectiveness of our internal control over financial reporting based on the framework in Internal Control-Integrated Framework (2013), updated and reissued by the Committee of Sponsoring Organizations, or the COSO Framework. Based on our evaluation under the COSO Framework, our management concluded that our internal control over financial reporting was not effective as of December 31, 2014. In connection with the above assessment, Retrophin management identified a material weakness in the control environment relating to a certain member of senior management who did not demonstrate the appropriate level of control consciousness and, therefore, did not demonstrate a positive tone at the top of the organization and 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 granting and accounting for equity awards.

During 2014 and 2015, our management has taken the following actions that materially affect, or are reasonably likely to materially affect, our internal control over financial reporting and to remediate the material weaknesses described above.

- We have implemented a new accounting system which allows for us to generate data in a form and format that facilitates the timely analysis of information needed to produce accurate financial reports.
- We have hired additional staff with expertise in applying complex accounting and financial reporting and disclosure rules required under GAAP and SEC reporting regulations.
- We have hired additional staff to assist in segregating duties.
- Effective October 2014, Gary A. Lyons and Jeffrey Meckler were appointed as independent members of the Board of Directors.
- On November 1, 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 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 more than 30 years of experience in accounting and finance, and pharmaceutical and biotechnology experience.
- 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.
- Effective March 31, 2015, Timothy Coughlin was appointed as an independent member of the Board of Directors.
- Effective April 1, 2015, Dr. John W. Kozarich was appointed as an independent member of the Board of Directors.
- Effective June 8, 2015, Jeffrey A. Meckler was appointed as the Chairman of the Board of Directors.

As discussed above, we have strengthened our management team and Board of Directors, and continue to evaluate our controls and processes associated with granting and accounting for equity awards in order to remediate the material weaknesses disclosed in our 2014 Form 10-K.

### **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 September 30, 2015 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## **PART II - OTHER INFORMATION**

### **Item 1. Legal Proceedings.**

On 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.

On September 19, 2014, a purported shareholder 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 plaintiff seeks, 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 parties have reached an agreement to settle the lawsuit. On October 19, 2015, Plaintiffs filed a motion seeking the Court’s approval of the settlement. If the Court approves the settlement, Mr. Shkreli will pay \$2,025,000 to the Company and will pay an additional \$625,000 to Plaintiffs to compensate them for their legal fees.

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 on June 26, 2015. Plaintiffs filed a consolidated opposition to the motions on July 27, 2015. Defendants filed their reply briefs in support of the motions on October 28, 2015. 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. The Company has also been cooperating with a parallel investigation by the U.S. Securities and Exchange Commission.

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 are currently selecting 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 September 30, 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.



**Item 1A. Risk Factors.**

The following risk factors do not reflect any material changes to the risk factors set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2014, other than the revisions to the risk factors set forth below with an asterisk (\*) next to the title. The following information sets forth risk factors that could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. If any of the following risks actually occur, our business, operating results, prospects or financial condition could be harmed. Additional risks not presently known to us, or that we currently deem immaterial, may also affect our business operations.

**Risks Related to the Commercialization of Our Products**

***\* The commercial success of Chenodal<sup>®</sup>, Thiola<sup>®</sup> and Cholbam<sup>®</sup> depends on them being considered to be effective drugs with advantages over other therapies.***

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

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

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

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

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

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

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

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 pressure on state budgets may result in states increasingly seeking to achieve budget savings through mechanisms that limit coverage or payment for our products. 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®.**

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 US 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 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. Under the Hatch-Waxman Amendments, a manufacturer may also submit 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.

**\* 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® and Thiola®, respectively. In addition, we plan to rely on a third party for manufacturing of 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 ("EMA"). Our dependence on third parties for the manufacture of our products may harm our profit margin on the sale of products and our ability to deliver products on a timely and competitive basis. If our third party manufacturers are unable to manufacture to specifications or in compliance with applicable regulatory requirements, our ability to commercialize our products will be adversely impacted and could affect our ability to gain market acceptance for our products and negatively impact our revenues.

We currently have no in-house distribution channels for Chenodal<sup>®</sup>, Thiola<sup>®</sup> or Cholbam<sup>®</sup> and we are dependent on a third-party specialty distributor, Dohmen Life Sciences Services to distribute such products. We rely on this distributor for all of our proceeds from sales of Chenodal<sup>®</sup>, Thiola<sup>®</sup> and Cholbam<sup>®</sup> 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 specialty distributor on substantially similar terms, distribution of Chenodal<sup>®</sup>, Thiola<sup>®</sup> and/or Cholbam<sup>®</sup> could become disrupted, resulting in lost revenues, provider dissatisfaction, and/or patient dissatisfaction.

#### ***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 and/or RE-024 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 has filed a U.S. IND and initiated 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, 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.

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;

- 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. FDA approval once obtained, may be withdrawn. If the regulatory approval for Thiola<sup>®</sup>, Chenodal<sup>®</sup> and/or Cholbam<sup>®</sup> 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<sup>®</sup>, 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 RE-034 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 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 the Development and Commercialization of our Products and Product Candidates

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

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

***\* 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 do not currently have patent protection for certain of our 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 pending application covering methods of treating PKAN by administering RE-024 has been allowed by the U.S. Patent and Trademark Office and is scheduled to issue as U.S. Patent No. 9,181,286 on November 10, 2015. 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, or 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

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.

***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 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.

***\* 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<sup>®</sup> and proprietary position with respect to sparsentan and RE-024 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.

***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.

***\*Any product for which we obtain marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.***

Any product for which we obtain marketing approval, along with the manufacturing processes and facilities, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and comparable regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if we obtain regulatory approval of a product, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product. We also may be subject to state laws and registration requirements covering the distribution of our products. Later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:



- restrictions on such products, manufacturers or manufacturing processes;
- warning letters;
- withdrawal of the products from the market;
- our ability to obtain and maintain marketing approvals from the FDA or similar regulatory authorities outside the United States;
- refusal to approve pending applications or supplements to approved applications that we submit;
- voluntary or mandatory recall;
- fines;
- suspension or withdrawal of regulatory approvals or refusal to approve pending applications or supplements to approved applications that we submit;
- refusal to permit the import or export of our products;
- product seizure or detentions;
- injunctions or the imposition of civil or criminal penalties; and
- adverse publicity.

If we, or our suppliers, third-party contractors, clinical investigators or collaborators are slow to adapt, or are unable to adapt, to changes in existing regulatory requirements or adoption of new regulatory requirements or policies, we or our collaborators may lose marketing approval for our products when and if any of them are approved, resulting in decreased revenue from milestones, product sales or royalties.

***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 into law the Patient Protection and Affordable Care Act and the Health Care Education Reconciliation Act (collectively, the "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. Although it is too early to determine the full effect of the Health Care Reform Law, the law appears likely to continue the 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:

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

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

***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 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 has no 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 expect to experience significant growth in the number of our employees and the scope of our operations. We began 2014 with 26 employees and ended the year with approximately 110 employees having added sales and marketing, compliance and legal functions in addition to expansion of all functions to support a commercial organization. As of September 30, 2015, we had approximately 126 employees. 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 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, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors.

***\*For the years ending December 31, 2013 and 2014, our management identified internal control deficiencies, which our management believed constituted material weaknesses. Any future material weaknesses or deficiencies in our internal control over financial reporting could harm stockholder and business confidence on our financial reporting, our ability to obtain financing and other aspects of our business.***

In connection with the preparation of our audited financial statements for the year ended December 31, 2014 we concluded that a material weakness existed in internal control over financial reporting. Specifically, as of December 31, 2014, our management concluded that the management of and accounting for equity awards and consulting agreements controls were not effective. On February 19, 2015, the Company's board of directors concluded that as a result of the errors related to such consulting agreements, the financial statements contained in the September 30, 2013 third quarter Form 10-Q and the Annual Report on Form 10-K for the year ended December 31, 2013 (the 2013 Form 10-K) should no longer be relied upon. The Company has corrected such errors, including any related disclosures, in this Quarterly Report on Form 10-Q, and we restated these periods in amendments to the September 30, 2013 third quarter Form 10-Q and 2013 Form 10-K that have been filed with the SEC. The Company believes that the errors related to such consulting agreements in the 2014 Forms 10-Q do not cause the financial statements contained therein to be misleading, and therefore such financial statements can still be relied upon. The Company has corrected such errors, including any related disclosures, in this Quarterly Report on Form 10-Q.

As of December 31, 2014, we carried out an assessment of the effectiveness of our internal control over financial reporting based on the framework in Internal Control-Integrated Framework (2013), updated and reissued by the Committee of Sponsoring Organizations, or the COSO Framework. Based on our evaluation under the COSO Framework, our management concluded that our internal control over financial reporting was not effective as of December 31, 2014. In connection with the above assessment, Retrophin management identified a material weakness in the control environment relating to a certain member of senior management who did not demonstrate the appropriate level of control consciousness and, therefore, did not demonstrate a positive tone at the top of the organization and 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. In the first three quarters of 2015 management implemented additional controls to remediate the material weakness disclosed in our Annual Report on Form 10-K for the year ended December 31, 2014 associated with the control environment, including strengthening our management team and implementing additional controls and processes associated with granting and accounting for equity awards.

Additionally, as of December 31, 2013, we had identified certain matters that constituted material weaknesses in our internal controls over financial reporting, including the fact that we (i) have experienced difficulty in generating data in a form and format

that facilitates the timely analysis of information needed to produce accurate financial reports, (ii) have experienced difficulty in applying complex accounting and financial reporting and disclosure rules required under GAAP and the SEC reporting regulations, and (iii) have limited segregation of duties. Although we are committed to continuing to improve our internal control processes, and although we will continue to diligently and vigorously review our internal control over financial reporting, any control system, regardless of how well designed, operated and evaluated, can provide only reasonable, not absolute, assurance that its objectives will be met. Management is in the process of taking the steps as outlined in Item 9A to remediate the December 31, 2014 material weaknesses. Therefore, we cannot be certain that, in the future, additional material weaknesses or significant deficiencies will not exist or otherwise be discovered. If our efforts to address the weakness identified are not successful, or if other deficiencies occur, these weaknesses or deficiencies could result in misstatements of our results of operations, restatements of our financial statements, a decline in our stock price and investor confidence or other material effects on our business, reputation, financial condition or liquidity.

***\*We have incurred operating losses since our inception. We expect to incur operating losses for the foreseeable future and may never achieve or maintain profitability.***

Management believes that we will continue to incur losses for the immediate future. For the year December 31, 2014, the Company generated sales revenue for the first time, and is trying to achieve a sustained positive cash flow from operations. 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 sustain our operations until we can achieve profitability and positive cash flows from operating activities, if ever.

At September 30, 2015, we had working capital surplus of approximately \$198.0 million and our accumulated deficit amounted to \$62.7 million. As of September 30, 2015 and December 31, 2014, our stockholders' equity was \$291.0 million and our stockholders deficit was \$37.3 million, respectively. Our net income for the three months ended September 30, 2015 was \$105.6 million compared to a net loss of \$18.0 million for the three months ended September 30, 2014, primarily due to the gain of \$140.0 million resulting from the sale of the Pediatric PRV. Our net income for the nine months ended September 30, 2015 was \$119.7 million compared to a net loss of \$81.9 million for the nine months ended September 30, 2014, primarily due to the gain of \$140.0 million resulting from the sale of the Pediatric PRV, and the change in the fair value of derivative instruments. Net cash used in operating activities was \$1.4 million and net cash provided by operating activities was \$2.0 million for the three and nine months ended September 30, 2015, respectively, compared to net cash used in operating activities of \$15.5 million and \$36.8 million in the three and nine months ended September 30, 2014, respectively. Operations since inception have been funded primarily with the proceeds from equity and debt financings and beginning in March 2014 from revenue from our marketed products. As of September 30, 2015, we had cash, cash equivalents and marketable securities of \$235.3 million. We will continue to fund operations from cash on hand, product revenues, and through the similar sources of capital previously described. We can give no assurance that such capital will be available to us on favorable terms or at all. If we are unable to raise additional funds in the future on acceptable terms, or at all, we may be forced to curtail our development activities. In addition we could be forced to delay or discontinue product development, and forego attractive business opportunities. Any additional sources of financing will likely involve the sale of our equity securities, which will have a dilutive effect on our stockholders.

We have devoted substantially all of our efforts to research and development, specifically our preclinical development activities. We have not completed development of any drugs. We expect to continue to incur significant and increasing operating losses for at least the next several quarters and we are unable to predict the extent of any future losses. 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<sup>®</sup> 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 become and remain profitable, 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.

***\*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 will continue to incur losses for the immediate future. For the year December 31, 2014, the Company generated revenue and is trying to achieve a sustained positive cash flow from operations. 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 sustain our operations until we can achieve profitability and positive cash flows from operating activities, if ever. 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;
- 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. Stock prices of many pharmaceutical companies have fluctuated in a manner unrelated or disproportionate to the operating performance of those 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.

***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 aggregate annual limit of coverage amount under these policies expressed in United States dollars is approximately \$5.0 million, and these policies are also subject to per claim deductibles. 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. In addition, we received a subpoena relating to a criminal investigation by the U.S. Attorney for the Eastern District of New York and are cooperating with this investigation and a parallel investigation by the Securities and Exchange Commission. While we are not named as a defendant or otherwise a target of these proceedings, such proceedings could result in substantial costs and significant adverse impact on our reputation and divert management's attention and resources, which could have a material adverse effect on our business, operating results or financial condition.

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

We are subject to significant ongoing regulatory obligations, such as safety reporting requirements and additional post-marketing obligations, including regulatory oversight of the promotion and marketing of our products. In addition, the manufacture, quality control, labeling, packaging, safety surveillance, adverse event reporting, storage, 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 to apply to arrangements that pharmaceutical companies have with prescribers, purchasers and formulary managers. 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 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 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, and imprisonment. Because of the breadth of these laws, it is possible that some of our business activities could be subject to challenge under one or more of these laws. Such a challenge, irrespective of the underlying merits of the challenge or the ultimate outcome of the matter, could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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

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

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

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

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

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

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

### **Risks Related to our Indebtedness and Investments**

***\*Our substantial indebtedness could adversely affect our financial condition.***

As of September 30, 2015, we had approximately \$46 million of total debt outstanding, classified as long term. The total debt outstanding as of September 30, 2015 related to a Note Purchase Agreement dated May 29, 2014 relating to the private placement of \$46.0 million aggregate senior secured notes (the "Notes"). As a result of our substantial indebtedness, a significant 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 substantial indebtedness pursuant to the Notes could have important consequences. For example, it could:

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

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

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

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

- failure to pay (for more than 30 days) interest when due;
- failure to pay principal when due;
- failure to deliver shares of Common Stock upon conversion of a Note;
- failure to provide notice of a fundamental change;
- acceleration on 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.

***\*The Notes rank junior to any of our secured indebtedness.***

The Notes are our general unsecured obligations; they are not secured by any of our assets or those of our subsidiaries. The Notes effectively rank junior to any secured indebtedness, including and any secured indebtedness that we may incur. In the event of our bankruptcy, liquidation, reorganization or other winding up, our assets that secure debt will be available to pay obligations on the Notes only after all debt under such secured debt, if any, has been repaid in full from such assets. As a result, it is likely that there would not be sufficient assets remaining to pay amounts due on any or all the Notes then outstanding. In addition, the terms of the Notes allow us to secure unlimited amounts of debt with our assets, all of which would be effectively senior to the Notes to the extent of the value of such assets.

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

None.

**Item 3. Defaults Upon Senior Securities.**

None.

**Item 4. Mine Safety Disclosures.**

Not applicable.

**Item 5. Other Information.**

None.

**Item 6. Exhibits**

(a) Exhibits

3.1	Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to Amendment No. 2 to the Company's General Form for Registration of Securities on Form 10-12G, filed with the SEC on October 28, 2010).
3.2	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
3.3	Amended and Restated Bylaws of the Company (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
4.1	Form of 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	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).
4.8	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	Amendment No. 4 to Sublicense Agreement dated as of September 17, 2015, between Retrophin Inc. and Ligand Pharmaceuticals Incorporated**
10.2	Addendum to Trademark License and Supply Agreement, dated October 19, 2015, by and between to Company and Mission Pharmacal
10.3	2015 Retrophin Inc. Executive/Designated Officer Annual Bonus Plan (incorporated by reference to Exhibit 99.1 to the Company's current report in Form 8-K filed with the SEC on July 29, 2015)
31.1	Chief Executive Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 *
31.2	Chief Financial Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 *
32.1	Chief Executive Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002 *
32.2	Chief Financial Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002 *
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Taxonomy Extension Presentation Linkbase Document

\* Filed herewith.

Confidential treatment has been requested with respect to certain portions of this exhibit. Omitted parties have filed separately with the SEC.

**SIGNATURES**

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

Date: November 6, 2015

**RETROPHIN, INC.**

By: /s/ Stephen Aselage

Name: Stephen Aselage

Title: Chief Executive Officer

By: /s/ Laura Clague

Name: Laura Clague

Title: Chief Financial Officer

## AMENDMENT NO. 4 TO SUBLICENSE AGREEMENT

**THIS AMENDMENT NO. 4 TO SUBLICENSE AGREEMENT** (the "**Amendment**") is made and entered into as of September 17, 2015 ("**Amendment Effective Date**") and amends the Sublicense Agreement effective as of February 16, 2012, as amended pursuant to that certain Amendment to Sublicense Agreement dated December 11, 2012, Amendment No. 2 to Sublicense Agreement dated January 7, 2013, and Amendment No. 3 to Sublicense Agreement dated February 27, 2015 (the "**Sublicense Agreement**") by and between Ligand Pharmaceuticals Incorporated, a corporation organized under the laws of Delaware and having a place of business at 11119 North Torrey Pines Road, Suite 200, La Jolla, CA, 92037 and its wholly owned subsidiary, Pharmacoepia, LLC (as successor in interest to Pharmacoepia Drug Discovery Inc.) ("**PCOP**"), a limited liability company organized under the laws of Delaware and having a place of business at 11119 North Torrey Pines Road, Suite 200, La Jolla, CA, 92037 (collectively, Ligand Pharmaceuticals Incorporated and PCOP shall be known as "**Ligand**") and Retrophin, Inc., a corporation organized under the laws of Delaware and having a place of business at 12255 El Camino Real, San Diego, CA 92130 ("**Retrophin**").

### BACKGROUND

**WHEREAS** Ligand and Retrophin have previously entered into the Sublicense Agreement pursuant to which Ligand sublicensed to Retrophin rights under the License Agreement dated March 27, 2006 between PCOP and Bristol-Myers Squibb Company (the "Upstream License"); and

**WHEREAS**, Ligand and Retrophin desire to amend certain terms of the Sublicense Agreement and the Upstream Agreement as set forth herein.

**NOW, THEREFORE**, in consideration of the foregoing premises and the mutual covenants contained herein, the Parties, intending to be legally bound, agree as follows:

1. **Capitalized Terms.** The capitalized terms used herein and not otherwise defined shall have the same definitions as provided in the Sublicense Agreement
2. **Amendments to Milestone Payments.**
  - a) **Development Milestone Payments.** Table 1 of Section 8.2.1 of the Agreement is hereby amended in its entirety as follows:

Milestone Event	Milestone Payment
Execution of Agreement	\$1.15 million
[...***...]	[...***...]

**\*\*Confidential Treatment Requested**

	[...***...]- <sup>1</sup>
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]

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<sup>-1</sup> If the Second Milestone and any Additional Payment is not received by Ligand on or before [...\*\*\*...], Ligand shall have the right to [...\*\*\*...] by providing written notice to Retrophin, notwithstanding [...\*\*\*...]. In addition, and for clarity, the provisions of [...\*\*\*...] shall be operative, including, without limitation, the provisions of subsections [...\*\*\*...]

**\*\*Confidential Treatment Requested**



[... ** ...]	[... *** ...]
[... *** ...]	[... *** ...]
[... *** ...]	[... *** ...]
[... *** ...]	[... *** ...]

b) Section 8.10 of the Sublicense Agreement is hereby deleted in its entirety.

**3. Consideration.** Retrophin shall pay Ligand (i) \$850,000 in consideration for the amendments set forth in this Amendment, and (ii) \$150,000 for the efforts to amend the Upstream License Agreement in accordance with Section 4 of this Amendment, in each case such payment shall be non-refundable and shall be made within 5 days of execution of this Amendment by both parties.

**4. Efforts to Amend Upstream License Agreement.**

(a) Ligand will use reasonable best efforts to obtain a waiver of Sections 3.1 and 13.2.5 by BMS for the Asia Pacific Region. “Asia Pacific Region” means Japan, China, S. Korea, Taiwan, Thailand and Vietnam.

(b) Ligand will use reasonable best efforts to obtain BMS’ agreement to the standby license provided by Section 2.2.2(v) in which event, Section 2.2.2(v) would be amended substantially in the form of the following language:

“...provided, that, that such sublicensed rights shall not terminate if, as of the effective date of termination by BMS under Section 13.2, the Sublicensee is not in material default under its license agreement with Ligand in which case Sublicensee will assume all of Ligand’s rights and obligation under this Sublicense Agreement and be bound directly to BMS substituting Sublicensee for Ligand and subject to the payment to Ligand of all royalties and milestones under the sublicense agreement to the extent they exceed payments due to BMS under this Sublicense Agreement and payment to BMS of all royalties and milestones under this Upstream Agreement.”

(c) Ligand will use reasonable best efforts to obtain BMS’s agreement to the following amendments to the termination provisions of the Upstream Agreement.

i. Section 13.4 (b) of the Upstream Agreement amended to read as set forth below:

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“[...\*\*\*...]”

ii. Section 13.4(f) amended as set forth below:

“Ligand will [...\*\*...]”

iii. Section 13.4(i) deleted.

(d) For the avoidance of doubt, any such efforts by Ligand made under this Sublicense Agreement shall not require Ligand to pay BMS any fee or concede any existing rights, but rather shall solely involve the use of logic and reason to seek to persuade BMS.

## **5. Amendments to Sublicense Agreement.**

a) For the avoidance of doubt, none of the following amendments to the Sublicense Agreement are intended to cause a breach of the Upstream Agreement and any amendment that would otherwise cause such a breach shall be null and void ab initio.

b) Section 1 of the Sublicense Agreement is hereby amended to include the following:

“1.70 “Asia Pacific Region” means Japan, China, S. Korea, Taiwan, Thailand and Vietnam.”

c) Section 2.2.2 (vi) is hereby revised as set forth below:

“...provided however, that such sublicensed rights shall not terminate if, as of the effective date of termination by Ligand under Article 13, the Sublicensee is not in material default under its license agreement with Retrophin in which case Sublicensee will assume all of Retrophin’s rights and obligation under this Sublicense Agreement and be bound directly to Ligand respectively substituting Sublicensee for Retrophin and subject to the payment to Retrophin of all royalties and milestones under the sublicense

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agreement to the extent they exceed payments due to Ligand under this Sublicense Agreement and payment to Ligand of all royalties and milestones under this Sublicense Agreement to the extent they exceed payments due to BMS under the Upstream Agreement.”

d) Section 3.2 of the Sublicense Agreement is hereby amended to include the following:

“3.2.4 The provisions of Sections 3.2.1 and 3.2.2 shall not apply within the Asia Pacific Region.”

e) Section 13.1.1 is hereby amended to add at the beginning of the first sentence “Subject to Section 13.7...”

f) Section 13.2.6 is hereby deleted.

g) Section 13.3 is hereby amended to add prior to the first sentence:

“Retrophin may terminate this Agreement for convenience upon [...\*\*...] ([...\*\*\*...]) days prior written notice to Ligand and all of the provisions of Section 13.4 will survive termination of this Agreement pursuant to this Section 13.3.”

h) The following amendments will be effective (i) as between Ligand and Retrophin at a time when there is no breach claimed by BMS under the Upstream Agreement, and/or (ii) at any time upon BMS’s agreement to amend or waive the applicable sections of the termination provisions in the Upstream Agreement;

a. Section 13.4(b) is hereby amended as set forth below:

“[...\*\*\*...]”

b. Section 13.4(f) amended as set forth below:

“Retrophin will [...\*\*...]”

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[...\*\*...]

c. Section 13.4(i) deleted.

**6. Further Agreements.**

- a) Ligand further agrees that it will not, by act or omission, cause the termination of the Upstream Agreement provided however Ligand may terminate the Upstream Agreement for good cause with Retrophin's prior written consent, not to be unreasonably withheld. Upon receipt by Ligand of any notice of default or any event that could likely lead to termination of the Upstream Agreement, Ligand will promptly notify Retrophin and work with Retrophin to effect cure of the default or concession with BMS.
- b) To the extent BMS shall not agree to the amendments proposed in Section 4 above, Ligand will, to the extent it does not cause a default under the Upstream Agreement, work with Retrophin in good faith and without further consideration and without refund of payments made hereunder to achieve the objectives contemplated by this Amendment by making further efforts to seek agreement from BMS.

**7. No Other Amendments.** Except as provided herein, the Sublicense Agreement shall continue in full force and effect.

**8. Governing Law.** This Amendment shall be governed by, enforced, and shall be construed in accordance with the laws of the State of New York without regard to its conflicts of law provisions.

**9. Counterparts.** This Amendment may be executed in counter-parts with the same effect as if both Parties had signed the same document. All such counterparts shall be deemed an original, shall be construed together and shall constitute one and the same instrument.

*[Signature Page Follows]*

**\*\*Confidential Treatment Requested**

**IN WITNESS WHEREOF**, the Parties have executed this Amendment to Sublicense Agreement through their duly authorized representatives to be effective as of the Amendment Effective Date.

**LIGAND PHARMACEUTICALS RETROPHIN, INC.  
INCORPORATED**

By: /s/ Charles Berkman By: /s/ Laura Clague

Name: Charles Berkman Name: Laura Clague

Title: VP, General Counsel & Secretary Title: Chief Financial Officer

SIGNATURE PAGE TO AMENDMENT TO SUBLICENSE AGREEMENT



PHARMACAL

Exhibit 10.2

October 15, 2015

Stephen Aselage  
CEO  
Retrophin Inc.  
12255 El Camino Real, Suite 250 San Diego, CA 92130

Re: Addendum to Trademark License and Supply Agreement dated May 28, 2014 Dear Stephen,

As you are aware, Retrophin has requested that Mission Pharmacal build a significant safety stock of tiopronin, the active pharmaceutical ingredient (API) in THIOLA. The safety stock is requested to insure that there is no interruption in the supply of THIOLA and to prepare for a potential development project, whereby THIOLA may be reformulated. Due to the financial burden of purchasing such large quantities of tiopronin prior to firm production orders, Mission is requesting immediate reimbursement for the safety stock purchases.

In order for Mission to execute on the acquisition of safety stock, Retrophin will need to submit an agreed upon annual plan of API purchases. Mission will then work with the approved supplier to schedule production and purchases. The Annual Plan of API purchases is attached to this letter agreement and agreed upon by the parties.

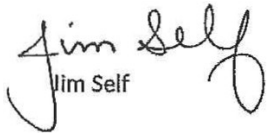
On Retrophin's behalf, Mission will purchase the agreed quantities of tiopronin safety stock. Mission requests that Retrophin issue a detailed demand forecast, outlining the quantities required for safety stock. Mission will then work with the supplier to order the requested API. Mission will prepare a purchase order detailing the amount of API to be ordered, the purchase price, and the total amount due to the supplier. Mission will then submit the purchase order and a detailed invoice to Retrophin for approval. The sum of the Mission invoice will be the total amount due from the prepared purchase order plus a 5% administration fee due to Mission. Following Retrophin's review and approval of the submitted purchase order and invoice, Retrophin will submit full payment for the invoice via wire transfer to Mission. Following receipt of Retrophin's approval and payment, Mission will issue a purchase order with the supplier. Mission will cooperate with Retrophin and its auditors for the conduct of physical inventory counts of the safety stock as needed in order for Retrophin to fulfill its public company accounting and reporting requirements.

Once the API safety stock material is put into production by Mission to fulfill Retrophin's firm production orders for finished market packages, Mission will invoice Retrophin for the finished market packages per the terms of the Supply Agreement. The invoice for the firm order of finished market packages will be at the agreed upon Supply Price for actual packages delivered, less a credit for the actual cost incurred for the pre-purchase of the API Safety Stock. The 5% administration fee will not be credited to the invoice.

This addendum will be governed by the terms and conditions of the Trademark License and Supply Agreement dated May 2014 (as amended) between Mission Pharmacal Company and Retrophin, Inc.

If Retrophin is in agreement with the terms proposed herein, please sign below where indicated and return to the attention of Mr. Lee Cusenbary via email at [lee.cusenbary@missionpharmacal.com](mailto:lee.cusenbary@missionpharmacal.com), with copy to Jim Self at [jim.self@missionpharmacal.com](mailto:jim.self@missionpharmacal.com).

Respectfully,

  
Jim Self

Cc: Lee Cusenbary, Tom Dooley, Chris Huggins, Bennett Kennedy, Darryl Johnson, Jon Taylor

**Agreed and Approved:**

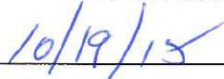
**Signature:**

  
\_\_\_\_\_

**By:**

Stephen Aselage  
CEO  
Retrophin Inc.

**Date:**

  
\_\_\_\_\_



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

I, Stephen Aselage, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Retrophin, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 6, 2015

/s/ Stephen Aselage

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Stephen Aselage

Chief Executive Officer

(Principal Executive Officer)



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

I, Laura Clague, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Retrophin, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 6, 2015

/s/ Laura Clague

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Laura Clague

Chief Financial Officer

(Principle Financial Officer)

**CERTIFICATION OF  
CHIEF EXECUTIVE OFFICER  
PURSUANT TO 18 U.S.C. SECTION 1350  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the accompanying Quarterly Report on Form 10-Q of Retrophin, Inc. (the "Company"), for the period ending September 30, 2015 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

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

Date: November 6, 2015

/s/ Stephen Aselage

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Stephen Aselage

Chief Executive Officer

(Principal Executive Officer)

**CERTIFICATION OF  
CHIEF FINANCIAL OFFICER  
PURSUANT TO 18 U.S.C. SECTION 1350  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the accompanying Quarterly Report on Form 10-Q of Retrophin, Inc. (the "Company"), for the period ending September 30, 2015 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

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

Date: November 6, 2015

/s/ Laura Clague

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Laura Clague

Chief Financial Officer

(Principal Financial Officer)