Retrophin, Inc.
Form 424B1
January 13, 2014
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Filed Pursuant to Rule 424(b)(1)
Registration No. 333-192936
PROSPECTUS
4,705,882 Shares

Retrophin, Inc.
Common Stock

We are offering 4,705,882 shares of our common stock. All of the shares of common stock are being sold by us.
Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "RTRX." Prior to this offering, our common stock has been quoted on the OTC QB market under the symbol "RTRX." On January 9, 2014, the last reported sale price of our common stock on the OTC QB market was $8.50 per share.
We are an "emerging growth company" as defined by the Jumpstart Our Business Startup Act of 2012 and, as such, we have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings. See "Prospectus Summary—Implications of Being an Emerging Growth Company."
Investing in our common stock involves a high degree of risk. Please read "Risk Factors" beginning on page 8.
Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Public Offering Price	$8.5000	$39,999,997
Underwriting Discounts and Commissions	$0.5525	$2,600,000
Proceeds to us, before expenses (1)	$7.9475	$37,399,997

(1)

- The underwriters will also be reimbursed for certain expenses incurred in this offering. See "Underwriting" for details.

Delivery of the shares of common stock is expected to be made on or about January 15, 2014. We have granted the underwriters an option for a period of 30 days to purchase an additional 705,882 of shares of our common stock. If the underwriters exercise the option in full, the total underwriting discounts and commissions payable by us will be $2,990,000, and the total proceeds to us, before expenses, will be $43,009,994.
Sole Book-Running Manager
Jefferies
Co-Managers

Roth Capital Partners

Ladenburg Thalmann & Co. Inc.

Summer Street Research Partners

Prospectus dated January 10, 2014

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This prospectus is part of a registration statement we filed with the Securities and Exchange Commission (the "SEC"). You should rely only on the information provided in this prospectus or in any free writing prospectus that we may provide you in connection with this offering. We have not, and the underwriters have not, authorized anyone to provide you with information different from that contained in this prospectus. If anyone provides you with different or inconsistent information, you should not rely on it. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities other than the common stock offered by this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any common stock in any circumstances in which such offer or solicitation is unlawful. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted.

Neither the delivery of this prospectus nor any sale made in connection with this prospectus shall, under any circumstances, create any implication that there has been no change in our affairs since the date of this prospectus or that the information contained by reference to this prospectus is correct as of any time after its date. The information in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of common stock. The rules of the SEC may require us to update this prospectus in the future.

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PROSPECTUS SUMMARY

The following summary is qualified in its entirety by the more detailed information appearing elsewhere in this prospectus. It may not contain all of the information that you should consider before investing in our common stock. You should read this entire prospectus carefully, including the "Risk Factors" and the financial statements and related included herein. This prospectus includes forward-looking statements that involve risks and uncertainties. See "Cautionary Note Regarding Forward-Looking Statements."

In this prospectus, 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.

Overview

We are a development-stage biopharmaceutical company focused on the development, acquisition and commercialization of therapies for the treatment of serious, catastrophic or rare diseases. We are developing Syntocinon TM Nasal Spray in the U.S. to assist initial postpartum milk ejection, which we refer to as aiding milk let-down, and for the treatment of Schizophrenia and Autism. Syntocinon Nasal Spray is currently marketed by Novartis and Sigma-Tau in Europe and other countries for aiding milk let-down. In addition, we are developing RE-034, a synthetic hormone analogue that is composed of the first 24 amino acids of the 39 amino acids contained in the naturally occurring adrenocorticotrophic hormone, or ACTH, for the treatment of Infantile Spasms and Nephrotic Syndrome. We are developing RE-024, a novel small molecule, as a potential treatment for pantothenate kinase-associated neurodegeneration, or PKAN. Also, we are developing sparsentan, formerly known as RE-021, a dual acting receptor antagonist of angiotensin and endothelin receptors, for the treatment of focal segmental glomerulosclerosis, or FSGS. We also have several additional programs in preclinical development, including RE-001, a therapy for the treatment of Duchenne Muscular Dystrophy.

Our Product Candidates

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Syntocinon Nasal Spray

Syntocinon (oxytocin nasal spray, USP) is our product candidate for aiding milk let-down and for the treatment of Schizophrenia and Autism. Syntocinon is currently sold in Europe and other countries by Novartis and Sigma-Tau to aid mothers experiencing problems with milk let-down. Oxytocin is a nonapeptide hormone synthesized by the brain and released by the pituitary gland.

Syntocinon Nasal Spray was an FDA-approved product for aiding milk let-down. Syntocinon Nasal Spray was voluntarily withdrawn from sale by Novartis Pharmaceutical Corporation, or Novartis, in 1997 for commercial reasons. On December 12, 2013, we secured a royalty-bearing license from Novartis to the U.S. rights for Syntocinon Nasal Spray, including the intellectual property to develop, manufacture, and sell the product in the United States.

Syntocinon Nasal Spray in Milk Let-Down

We intend to reintroduce Syntocinon to the U.S. market to assist initial postpartum milk ejection from the breasts. Disruption of oxytocin plays an important role in preventing the release of milk from the lactating breast. Numerous psychological and chemical stressors have been implicated in the inhibition of oxytocin release in new mothers resulting in impaired milk-ejection. There are currently no FDA-approved drugs for the treatment of milk let-down in the U.S. We believe that reintroduction of intranasal oxytocin would provide a convenient therapy for new mothers suffering from lactation deficiency.

Syntocinon Nasal Spray in Schizophrenia

We intend to develop Syntocinon as a potential treatment for Schizophrenia. Current pharmaceutical treatment is limited to powerful antipsychotics with serious side effects and compliance problems. According to the National Institute of Mental Health, approximately one percent of Americans suffer from Schizophrenia. Over the past four years, three randomized, double-blind, placebo-controlled, independent proof-of-concept schizophrenia trials were held. In all three trials, patients were highly symptomatic despite receiving therapeutic doses of an atypical antipsychotic. We believe that the findings of these studies suggest that intranasal oxytocin administered as an adjunct to subjects' antipsychotic drugs will improve positive and negative symptoms. We are partially funding a Phase 2 clinical study regarding the effects of oxytocin on the treatment of Schizophrenia. This trial is currently enrolling patients, and we expect approximately 143 patients to be enrolled. We expect results from this trial in the third quarter of 2014.

Syntocinon Nasal Spray in Autism Spectrum Disorders

We also plan to develop Syntocinon for the potential treatment of symptoms in patients with Autism Spectrum Disorders. Approximately one in fifty children in the U.S. suffers from Autism Spectrum Disorders according to the Center for Disease Control and Prevention. Risperidone and aripiprazole are the only approved treatments for the behavioral disturbances associated with Autism. Common adverse effects from these drugs include weight gain, sedation, and extrapyramidal symptoms. Recent small clinical studies suggest that oxytocin may improve social cognition and quality of life in patients with Autism. We believe that these studies support the development of Syntocinon for this indication. We intend to initiate a Phase 2 clinical study of Syntocinon for the treatment of Autism Spectrum Disorders in 2014.

RE-034 (Tetracosactide Zinc)

RE-034 is a synthetic hormone analog of the first 24 amino acids of the 39 amino acids contained in ACTH, formulated together with zinc. RE-034 exhibits the same physiological actions as endogenous ACTH by binding to all five melanocortin receptors (MCR), resulting in its anti-inflammatory and immunomodulatory effects. In 2014, we plan to submit an Investigational New Drug application, or IND, for RE-034 for the treatment of Infantile Spasms and Nephrotic Syndrome to the FDA.

RE-034 in Infantile Spasms

Infantile Spasms, or IS, also known as West syndrome, is a form of epileptic encephalopathy that begins in infancy. IS is considered a catastrophic form of epilepsy due to the difficulty in controlling seizures and normalization of electroencephalography in addition to strong association with sequelae of developmental delay and mental retardation. Commercially available ACTH formulations that are substantially similar to RE-034 have been shown to be an effective treatment of Infantile Spasms. We intend to initiate a Phase 3 clinical trial of RE-034 for the treatment of Infantile Spasms in 2014.

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RE-034 in Nephrotic Syndrome

We intend to initiate studies of RE-034 for the treatment of Nephrotic Syndrome, or NS. Nephrotic Syndrome is a kidney disorder that leads to proteinuria, a condition in which an excess of proteins are contained in a patient's urine. Long-term conventional immunosuppression therapies have been used effectively to induce remission of proteinuria; however, many patients with Nephrotic Syndrome will relapse after remission or are resistant to primary and secondary treatments. Commercially available ACTH formulations that are substantially similar to RE-034 have been shown to successfully induce remission of proteinuria in patients with Nephrotic Syndrome. We intend to initiate a Phase 3 clinical trial of RE-034 for the treatment of Nephrotic Syndrome in 2014.

RE-024

We are developing RE-024, a novel small molecule, as a potential treatment for PKAN. PKAN is the most common form of neurodegeneration with brain iron accumulation. Classic PKAN is a genetic disorder that is typically diagnosed in the first decade of life. Consequences of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. PKAN is estimated to affect 1 to 3 persons per million. PKAN typically manifests in childhood with a profound, progressive dystonia and is usually lethal. There are currently no viable treatment options for patients with PKAN. RE-024 is a phosphopantothenate prodrug replacement therapy with the goal of restoring the supply of this operative substrate in PKAN patients. A Phase 1 clinical trial of RE-024 is expected to begin in early 2014 under an emergency IND.

Sparsentan

Sparsentan, formerly known as RE-021, is an investigational therapeutic agent which acts as both a potent angiotensin receptor blocker, or ARB, which is a type of drug that modulates the renin-angiotensin-aldosterone system and is typically used to treat hypertension, diabetic nephropathy and congestive heart failure, as well as a selective endothelin receptor antagonist, or ERA, which is a type of drug that blocks endothelin receptors, preferential for endothelin receptor type A. We have secured a license to sparsentan from Ligand and Bristol-Myers Squibb (who referred to it as DARA). We are developing sparsentan as a treatment for FSGS. FSGS is a leading cause of end-stage renal disease and Nephrotic Syndrome. We are currently enrolling patients for a Phase 2 clinical study of sparsentan for the treatment of FSGS and we expect approximately 100 patients to be enrolled.

Our Strategy

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

- Expand our product pipeline by pursuing additional acquisitions of pharmaceutical products that have a profound impact on patients' lives;
- Focus on developing products to treat orphan or severe diseases;
- Develop a sustainable pipeline by employing disciplined decision criteria; and
- Evaluate the commercialization strategies on a product-by-product basis to maximize the value of each.

Risks Associated with Our Business

Our ability to implement our current business strategy is subject to numerous risks, as more fully described in the section entitled "Risk Factors" immediately following this prospectus summary. You should carefully consider all of the

information set forth in this prospectus and, in particular, should evaluate the specific factors set forth under “Risk Factors” and “Cautionary Note On Forward Looking Statements” in deciding whether to invest in our common stock. Among these important risks and uncertainties that could adversely affect our results of operations and business condition are the following:

- We have incurred significant losses since our inception and anticipate that we will continue to incur losses for the foreseeable future. We are a development stage company with no approved products, and no historical revenues, which makes it difficult to assess our future viability.

- Other companies may pursue similar strategies or initiate similar clinical studies.

- Our success is primarily dependent on the successful development, regulatory approval and commercialization of our product candidates, all of which are in early development.

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- Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies and trials may not be predictive of future trial results.

- The regulatory approval processes of the FDA and similar foreign authorities is lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

- If we fail to obtain additional financing, we may be unable to complete the development and commercialization of our product candidates, or continue our development programs. In addition, the report of our independent registered public accounting firm on our financial statements appearing at the end of this prospectus contains an explanatory paragraph stating that our recurring losses raise substantial doubt about our ability to continue as a going concern. Our commercial success depends upon attaining significant market acceptance of our product candidates, if approved, among physicians, patients and healthcare payors.

- We face significant competition from other biotechnology and pharmaceutical companies and our operating results will suffer if we fail to compete effectively.

- We depend on the performance of third parties, including contract research organizations and third-party manufacturers.

Implications of Being an Emerging Growth Company

We are an “emerging growth company” as defined in the Jumpstart Our Business Startups Act, or “JOBS Act.” For as long as we are an emerging growth company, unlike other public companies, we will not be required to:

- provide an auditor’s attestation report on management’s assessment of the effectiveness of our system of internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002;

- provide more than two years of audited financial statements or two years of management’s discussion and analysis;

- comply with any new requirements adopted by the Public Company Accounting Oversight Board, or the PCAOB, requiring mandatory audit firm rotation or a supplement to the auditor’s report in which the auditor would be required to provide additional information about the audit and the financial statements of the issuer;

- comply with any new audit rules adopted by the PCAOB after April 5, 2012, unless the SEC determines otherwise;

- provide certain disclosure regarding executive compensation required of larger public companies; or

- obtain shareholder approval of any golden parachute payments not previously approved.

We will cease to be an "emerging growth company" upon the earliest of (i) when we have $1.0 billion or more in annual revenues, (ii) when we have at least $700 million in market value of common stock held by non-affiliates, (iii) when we issue more than $1.0 billion of non-convertible debt over a three-year period, or (iv) the last day of the fiscal year following the fifth anniversary of our initial public offering.

In addition, Section 107 of the JOBS Act also 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. In other words, an emerging growth company 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.

Corporate Overview

For a description of our corporate background, please see "Corporate History" on page 52 of this prospectus. Our principal offices are located at 777 Third Avenue, 22nd Floor, New York, NY 10017. Our telephone number is (646) 837-5863. We also have offices in Cambridge, Massachusetts and San Diego, California. Our website address is www.retrophin.com. Our website and the information contained on, or that can be accessed through, the website will not be deemed to be incorporated by reference in, and are not considered part of, this prospectus. You should not rely on any such information in making your decision whether to purchase our common stock.

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THE OFFERING

Common stock offered by us in this offering

4,705,882 shares

Common stock outstanding after the offering

23,082,245 shares (23,788,127 shares if the underwriters' option to purchase 705,882 additional shares is exercised in full)

Underwriters' option to purchase additional shares

We have granted the underwriters an option to purchase up to 705,882 of additional shares of our common stock. This option is exercisable, in whole or in part, for a period of 30 days from the date of this prospectus.

Listing

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "RTRX."

Risk factors

Investing in our common stock involves a high degree of risk. See "Risk Factors."

In this prospectus, the number of shares of our common stock to be outstanding following this offering and other information based thereon is based on 18,376,363 shares of our common stock outstanding as of September 30, 2013 and assumes no exercise by the underwriters of their option to purchase additional shares.

The number of shares of our common stock outstanding following this offering and the other information based thereon does not reflect:

- 260,000 shares of our common stock issuable upon the exercise of options outstanding as of September 30, 2013, with a weighted average exercise price of $7.12 per share; and

- 4,462,426 shares of our common stock issuable upon the exercise of warrants outstanding as of September 30, 2013, with a weighted average exercise price of $5.14 per share.

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Summary Financial Data

We have derived the following summary financial data for the years ended December 31, 2012 and 2011 from our audited financial statements. The summary financial data for the nine months ended September 30, 2013 and 2012 and the balance sheet data as of September 30, 2013 have been derived from our unaudited interim financial statements. The unaudited interim financial results have been prepared on the same basis as the audited financial statements and reflect all adjustments necessary to fairly reflect our financial position as of September 30, 2013 and results of operations for the nine months ended September 30, 2013 and 2012. Our historical results are not necessarily indicative of the results that may be expected in the future. The following summary financial data should be read in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included in this prospectus.

(In thousands, except share and per share amounts)	**For the year ended December 31,**	**For the period from March 31, 2011 (inception) through December 31,**	**For the nine months ended September 30,**	
	2012	**2011**	**2013**	**2012**
Operating expenses:				
Compensation and related costs	$18,133,550	$2,227,203	$1,767,195	$ 8,371,481
Professional fees	8,494,583	556,287	4,392,673	7,761,899
Research and development	541,119	353,394	2,113,813	286,889
Selling, general and administrative	1,387,765	126,812	4,131,193	337,622
Technology license fee	1,700,000	—	100,000	—
Total operating expenses	30,257,017	3,263,696	12,504,874	16,757,891
Operating loss	(30,257,017)	(3,263,696)	(12,504,874)	(16,757,891)
Total other expense, net	(86,839)	(4,560)	(8,184,362)	(54,778)
Net loss	$(30,343,856)	$(3,268,256)	$(20,689,236)	$ (16,812,669)
Net loss per common share, basic and diluted	$(8.29)	$(1.59)	$(1.62)	$ (5.55)
Weighted average common shares outstanding, basic and diluted	3,662,114	2,053,402	12,797,714	3,027,468

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The table below presents our balance sheet as of September 30, 2013:

- on an actual basis; and

- on an as adjusted basis to give effect to the sale of shares of common stock in this offering at a public offering price of $8.50 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

	AS OF SEPTEMBER 30, 2013	
(In thousands)	**ACTUAL**	**AS ADJUSTED**
BALANCE SHEET DATA:		
Cash	$13,410	$ 50,010
Marketable securities, available-for-sale	2,957	2,957
Property and equipment, net	38	38
Total assets	21,365	57,965
Total current liabilities	27,169	27,169
Accumulated deficit	(54,301)	(54,301)
Total stockholders' (deficit) equity	(5,804)	30,796

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RISK FACTORS

Our business, as well as our common stock, are highly speculative in nature and involve a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information included herein, including the financial statements and related notes, before deciding to invest in our common stock. If any of the following risks actually occur, they could adversely affect our business, prospects, financial condition and results of operations. In such event, the market price of our common stock could decline and you could lose part or all of your investment. Accordingly, prospective investors should carefully consider, along with other matters referred to herein, the following risk factors in evaluating our business before purchasing any of our common stock.

Risks Related to Our Business

We are still in the development stage and have not generated any revenues.

From inception through September 30, 2013, we have incurred net losses of approximately $54.30 million and negative cash flows from operating activities of approximately $12.96 million. Because it takes years to develop, test and obtain regulatory approval for our treatments before they can be sold, we likely will continue to incur significant losses and cash flow deficiencies for the foreseeable future. Accordingly, we may never be profitable and, if we do become profitable, we may be unable to sustain profitability.

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.

Since inception, we have incurred significant operating losses. Our net loss attributable to common stockholders was $30.34 million for the year ended December 31, 2012. As of September 30, 2013 we had an accumulated deficit of $54.30 million. To date, we have financed our operations primarily by raising capital through private placements of our securities. 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:

- seek regulatory approval for Syntocinon for aiding milk let down and fund clinical trials for additional indications for Syntocinon;
- continue our ongoing preclinical development of RE-034,
- continue our ongoing preclinical development of RE-024 for the treatment of PKAN, and potentially begin clinical trials of RE-024;
- begin Phase 2 clinical development of sparsentan for the treatment of FSGS;
- continue our ongoing preclinical development activities of RE-001 for the treatment of DMD, and potentially begin clinical trials of RE-001;
- continue the research and development of additional product candidates;

- seek regulatory approval of Syntocinon for additional indications, RE-034, RE-024, sparsentan, RE-001 and additional product candidates;

- establish a sales and marketing infrastructure to commercialize products for which we may obtain regulatory approval; and

- add 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 never succeed in these activities and may never 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 you to lose a part or all of your investment.

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We are an early stage corporation. Our limited operating history makes it difficult to evaluate our current business and future prospects, and our profitability in the future is uncertain.

We commenced operations in 2011 and are a new, early stage company. As of the date hereof, we have not generated any revenues. Our operations to date have been limited to organizing and staffing our company, licensing and developing our technology, planning for clinical studies of sparsentan, developing a viable manufacturing route for RE-001, planning pre-clinical studies and limited clinical studies of RE-024 and RE-001. We have not yet demonstrated our ability to successfully begin or complete clinical trials, obtain regulatory approvals, manufacture a commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. In addition, we only recently began development of Syntocinon and RE-034. Consequently, any predictions you make about our future success or viability may not be as accurate as they would be if we had a longer operating history.

Our company faces 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. There can be no assurance that we will ever generate revenues from operations.

Moreover, even if 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 United States Food and Drug Administration, or 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.

We are subject to various laws and regulations, including “fraud and abuse” laws and anti-bribery laws, and a failure to comply with such laws and regulations or prevail in any litigation related to noncompliance could have a material adverse impact on our business, financial condition and results of operations and could cause the market value of our common stock to decline.

Pharmaceutical and biotechnology companies have faced lawsuits and investigations pertaining to violations of health care “fraud and abuse” laws, such as the federal False Claims Act, the federal Anti-Kickback Statute, the U.S. Foreign Corrupt Practices Act, or the FCPA, and other state and federal laws and regulations. We also face increasingly strict data privacy and security laws in the U.S. and in other countries, the violation of which could result in fines and other sanctions. The United States Department of Health and Human Services Office of Inspector General recommends and, increasingly, states require pharmaceutical companies to have comprehensive compliance programs and to disclose certain payments made to healthcare providers or funds spent on marketing and promotion of drug products. If we are in violation of any of these requirements or any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines, exclusion from federal healthcare programs or other sanctions.

The FCPA 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 many 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 U.S. and Canada. 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.

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We will need substantial additional 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 begin Phase 3 clinical studies of RE-034 and Phase 2 clinical studies of Syntocinon and sparsentan, and as we continue toward Phase 1 clinical studies of RE-024 and RE-001, 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.

We believe that our existing cash as of the date of this filing, together with the proceeds of this offering, and marketable securities, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months. 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:

- if approved by the FDA, our marketing and sales efforts for Syntocinon for aiding milk let-down;
- the progress and results of our pre-clinical and clinical studies of Syntocinon, RE-034, RE-024, sparsentan, RE-001, and other drug candidates;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and

- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

Any additional funds that we obtain may not be on terms favorable to us or our stockholders or may require us to relinquish valuable rights.

Until such time, if ever, as we generate stable product revenue to finance our operations, we expect to finance our cash needs through public or private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, foundations and government agencies. If we raise additional funds by issuing equity securities, our stockholders will experience dilution. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, and may include rights that are senior to the holders of our common stock. Any debt financing or additional equity that we raise may contain terms, such as liquidation and other preferences, which are not favorable to us or our stockholders. If we raise additional funds through collaboration and licensing arrangements with third parties, it may be necessary to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us or our stockholders.

Our management has identified internal control deficiencies, which our management believes constitute 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 period from March 11, 2011 (inception) through December 31, 2011 and the year ended December 31, 2012, we concluded that a material weakness existed in internal control over financial reporting and our disclosure controls. Specifically, our management concluded as of September 30, 2013 that our disclosure controls were not effective, as of such date, to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act was (i) recorded, processed, summarized and reported, within the time periods specified in the SEC rules and forms and (ii) accumulated and communicated to our management, including our Principal Executive Officer and Principal Financial Officer, as appropriate, to allow timely

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decisions regarding required disclosure. 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. 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, results of operations, financial condition or liquidity.

Our auditors have expressed doubt about our ability to continue as a going concern.

The Independent Registered Public Accounting Firm's Report issued in connection with our audited financial statements for the period from March 11, 2011 (inception) through December 31, 2011 and the year ended December 31, 2012 stated that "the Company, as a development stage enterprise, is subject to risks and uncertainties as to whether it will be able to raise capital and commence its planned operations. These conditions raise substantial doubt about the Company's ability to continue as a going concern." Because we have been issued an opinion by our auditors that substantial doubt exists as to whether it can continue as a going concern, it may be more difficult to attract investors. If we are not able to continue our business as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our financial statements, and it is likely that investors will lose all or a part of their investment.

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. We filed a U.S. patent application on RE-024 in April 2012, for which we received a notice of allowance from the United States Patent and Trademark Office in January, 2014. We have licensed composition of matter patents on sparsentan that expire in 2019. Currently we have no patent protection on Syntocinon, RE-034 or RE-001. We expect that in addition to the protection afforded by our patent filings that we will be able to obtain five years regulatory exclusively via the provisions of the Hatch-Waxman Amendments to the Federal Food, Drug, and Cosmetic Act, or FDC Act for products we develop based on a new chemical entity not previously approved by the FDA, and 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 filed a patent application in the United States on the composition of RE-024 as a treatment for pantothenate kinase associated neurodegeneration. Further, we have not filed for patent protection outside of the United States for RE-024. We cannot be certain that we will file for patent protection outside the United States, or that, even if we do, any patents(s) will be granted.

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We have negotiated a license agreement for the rights to DARA (PS433540), an ARB and ERA which we are initially using in connection with the treatment of FSGS and which we refer to as sparsentan and formerly referred to as RE-021, from Ligand Pharmaceuticals, Inc. ("Ligand" or "Ligand Pharmaceuticals"). We cannot be certain when or if we will file for patent protection for different indications, if we would be successful in obtaining these patents, or if we will be able to enforce these patents. If we are unsuccessful in obtaining patents for different uses of sparsentan, we may not be able to stop competitors from marketing similar products.

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 Invests 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 United States Patent and Trademark Office 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.

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, a pharmaceutical manufacturer may file an abbreviated new drug application, or 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 filing and reviewing) 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 protect its patents, approval of the ANDA is stayed for 30 months, or as lengthened or shortened by the court.

The composition of matter patents for Syntocinon have expired. Because Syntocinon has no regulatory exclusivity or listed patents, a competitor could at any time submit an ANDA or a Section 505(b)(2) NDA referencing Syntocinon and request immediate approval. The drug approval process is a confidential one, so we may not become aware of any new competitors until such ANDA or Section 505(b)(2) NDA has been approved by the FDA.

We license patent rights from third-party owners. If such owners do not properly or successfully obtain, maintain or enforce the patents underlying such licenses, our competitive position and business prospects will be harmed.

We have negotiated license agreements for the rights to Syntocinon Nasal Spray in the U.S. from Novartis and for the rights to sparsentan from Ligand Pharmaceuticals. We may enter into additional licenses to third-party intellectual property in the future. Our success will depend in part on the ability of our licensors to obtain, maintain and enforce patent protection for our licensed intellectual property, in particular, those patents to which we have secured or may secure exclusive rights. Our licensors may not successfully prosecute the patent applications to which we are licensed. Even if patents issue in respect of these patent applications, our licensors may fail to maintain these patents, may determine not to pursue litigation against other companies that are infringing these patents, or may pursue such litigation less aggressively than we would. Without protection for the intellectual property we license, other companies might be able to offer substantially identical products for sale, which could adversely affect our

competitive business position and harm our business prospects.

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If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We cannot be certain that we will be successful in maintaining the covenants required in our license agreements with Novartis, Ligand Pharmaceuticals or other third-party licensors, and we cannot be certain that we will be able to maintain these rights with beneficial terms.

If we are unable to protect the confidentiality of our proprietary information and know-how, the value of our technology and products could be adversely affected.

We seek to protect our know-how and confidential information, in part, by confidentiality agreements with our employees, corporate partners, outside scientific collaborators, sponsored researchers, consultants and other advisors. We also have confidentiality and invention or patent assignment agreements with our employees and our consultants. If our employees or consultants breach these agreements, we may not have adequate remedies for any of these breaches. In addition, our trade secrets may otherwise become known to or be independently developed by others. Enforcing a claim that a party illegally obtained and is using our trade secrets is difficult, expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States may be less willing to protect trade secrets. Costly and time consuming litigation could be necessary to seek to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We may become involved in infringement actions which are uncertain, costly and time-consuming and could have a material adverse effect on our business, financial condition and results of operations and could cause the market value of our common stock to decline.

The pharmaceutical industry historically has generated substantial litigation concerning the manufacture, use and sale of products and we expect this litigation activity to continue. As a result, we expect that patents related to our products will be routinely challenged, and our patents may not be upheld. In order to protect or enforce patent rights, we may initiate litigation against third parties. If we are not successful in defending an attack on our patents and maintaining exclusive rights to market one or more of our major products still under patent protection, we could lose a significant portion of sales in a very short period. We may also become subject to infringement claims by third parties and may have to defend against charges that we violated patents or the proprietary rights of third parties. If we infringe the intellectual property rights of others, we could lose our right to develop, manufacture or sell products, including our generic products, or could be required to pay monetary damages or royalties to license proprietary rights from third parties. The outcomes of infringement action are uncertain and infringement actions are costly and divert technical and management personnel from their normal responsibilities.

If we infringe or are alleged to infringe the intellectual property rights of third parties, it will adversely affect our business. Intellectual property disputes could require us to spend time and money to address such disputes and could be unsuccessful and/or limit our intellectual property rights.

Our research, development and commercialization activities, as well as any product candidates or products resulting from these activities, may infringe or be accused of infringing one or more claims of an issued patent or may fall within the scope of one or more claims in a published patent application that may subsequently issue and to which we do not hold a license or other rights. Third parties may own or control these patents or patent applications in the United States and abroad. These third parties could bring claims against us that would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages. Further, if a patent infringement suit were brought against us, we or they could be forced to stop or delay research, development, manufacturing or sales of the product or product candidate that is the subject of the suit.

No assurance can be given that patents do not exist, have not been filed, or could not be filed or issued, which contain claims covering our products, technology or methods. Because of the number of patents issued and patent applications filed in our field, we believe there is a risk that third parties may allege they have patent rights encompassing our products, technology or methods.

We are aware, for example, of United States patents, and corresponding international counterparts, owned by third parties that contain claims related to treating DMD using a direct protein replacement strategy. We also are aware of certain pending published patent applications (but no granted patents) in the United States, and corresponding

international counterparts, owned by third parties that contain claims related to the use of oxytocin (the active ingredient of Syntocinon) for the treatment of psychiatric disorders, including autism and schizophrenia. If such claims were to issue in a granted

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patent in their present form, we could be required to obtain a license. We may be unable to obtain such a license under commercially reasonable terms, or at all. If any third-party patents were to be asserted against us, we do not believe that our proposed products would be found to infringe any valid claim of these patents. If we were to challenge the validity of any issued United States patent in court, we would need to overcome a presumption of validity that attaches to every patent. This burden is high and would require us to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on infringement or validity. In order to avoid or settle potential claims with respect to any of the patent rights described above or any other patent rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or our future collaborators were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. This could harm our business significantly.

Others may sue us for infringing their patent rights or file nullity, opposition or interference proceedings against our patents, even if such claims are without merit, which would similarly harm our business. Furthermore, during the course of litigation, confidential information may be disclosed in the form of documents or testimony in connection with discovery requests, depositions or trial testimony. Disclosure of our confidential information and our involvement in intellectual property litigation could materially adversely affect our business.

There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the pharmaceutical and biotechnology industries. In addition to infringement claims against us, we may become a party to other patent litigation and other proceedings, including interference proceedings declared by the United States Patent and Trademark Office and opposition proceedings in the European Patent Office regarding intellectual property rights with respect to our products and technology. Even if we prevail, the cost to us of any patent litigation or other proceeding could be substantial.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from any litigation could significantly limit our ability to continue our operations. Patent litigation and other proceedings may also absorb significant management time.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. We try to ensure that our employees do not use the proprietary information or know-how of others in their work for us. However, we may be subject to claims that we or these employees have inadvertently or otherwise used or disclosed intellectual property, trade secrets or other proprietary information of any such employee's former employer. Litigation may be necessary to defend against these claims and, even if we are successful in defending ourselves, could result in substantial costs to us or be distracting to our management. If we fail to defend any such claims, in addition to paying monetary damages, we may jeopardize valuable intellectual property rights, disclose confidential information or lose personnel.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition by potential partners or customers in our markets of interest. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected.

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.

We face competition from pharmaceutical companies in the Schizophrenia, Autism Spectrum Disorders, IS, NS, FSGS and DMD indications and will likely face similar competition in other indications, including PKAN, because competition in the area of pharmaceutical products is intense. There are many companies, both public and private, including well-known pharmaceutical companies, which are engaged in the development of products for certain of the applications being pursued by Retrophin, such as Schizophrenia, Autism Spectrum Disorders, IS, NS, PKAN, FSGS

and DMD.

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For example, Questcor Pharmaceuticals, Inc.'s product H.P. Acthar Gel is a formula of ACTH that is approved by the FDA for the treatment of IS and NS. In addition, Apo Pharma Inc. and Treat Iron-Related Childhood-Onset Neurodegeneration ("TIRCON") are sponsoring clinical studies of Deferiprone as a potential treatment for PKAN. Also, we believe that TIRCON is working on a possible treatment for PKAN using pantethine derivatives.

Additionally, there are clinical studies underway evaluating possible treatments for FSGS. For example, Sanofi (Genzyme) is engaged in a Phase 2 clinical study of Fresolimumab to treat FSGS, and Sunnybrook Medical Center has announced plans for a Phase 2 clinical study of Rituxan to treat FSGS. Also, Fibrogen is developing an anti-Connective Tissue Growth Factor (CTGF) antibody as a possible treatment for FSGS.

The following biotechnology and pharmaceutical companies are working on developing potential treatments for DMD and have products which are currently in or have completed the following clinical stages: GlaxoSmithKline/Prosensa and Santhera/Takeda (Phase 3); Acceleron Pharma/Shire, Sarepta Therapeutics, Phrixus, Prosensa and PTC Therapeutics (Phase 2); and Sarepta Therapeutics and Tivorsan Pharmaceuticals and possibly others (Preclinical). Additionally, several FDA approved drugs for other indications are being tested in clinical trials for DMD, including prednisone, sildenafil citrate (sold under the trademark Viagra, among others) and IGF-1.

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 Retrophin, 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. If we are able to establish and maintain a significant proprietary position with respect to our products, competition likely will depend primarily on the effectiveness and ease of administration and product compliance as compared to alternative products. The industry in which we compete is characterized by extensive research and development efforts and rapid technological progress. Although we believe that our proprietary position may give us a competitive advantage with respect to sparsentan and RE-024, new developments are expected to continue and there can be no assurance that discoveries by others will not render such potential products noncompetitive.

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

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

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- 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 current good manufacturing practices, or 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 competiveness.

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

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

We rely on the manufacturers of our product candidates to purchase from third-party suppliers the materials necessary to produce the compounds for our preclinical and clinical studies and will 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, 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.

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We rely on third parties to conduct certain preclinical development activities and our clinical trials and those third parties may not perform satisfactorily, including failing to meet established deadlines for the completion of such activities and trials.

We do not currently operate any laboratory facilities. We do not independently conduct any physical preclinical development activities of our product candidates, such as efficacy and safety studies in animals, or clinical trials for our product candidates. We rely on, or work in conjunction with, third parties, such as contract research organizations, medical institutions and clinical investigators, to perform these functions. Our reliance on these third parties for preclinical and clinical development activities reduces our control over these activities. We are responsible for ensuring that each of our pre-clinical development activities and our clinical trials is conducted in accordance with the applicable general investigational plan and protocols and in compliance with appropriate government regulations, however, we have no direct control over these researchers or contractors (except by contract), as they are not our employees. Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices, or GCP, for conducting, recording and reporting the results of our preclinical development activities and our clinical trials to assure that data and reported results are credible and accurate and that the rights, safety and confidentiality of trial participants are protected. For our commercial products, we are required to comply with cGMP. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, comply with cGMPs, conduct our preclinical development activities or our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Moreover, these third parties may be bought by other entities or they may go out of business, thereby preventing them from meeting their contractual obligations.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

We currently do not have an organization for the sales, marketing and distribution of pharmaceutical products and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any products that may be approved, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services.

We may co-promote our product candidates in various markets with pharmaceutical and biotechnology companies in instances where we believe that a larger sales and marketing presence could expand the market or accelerate penetration. If we do enter into arrangements with third parties to perform sales and marketing services, our product revenues may be lower than if we directly sold and marketed our products and any revenues received under such arrangements will depend on the skills and efforts of others. However, we may not be successful in entering into distribution arrangements and marketing alliances with third parties. Our failure to enter into these arrangements on favorable terms could delay or impair our ability to commercialize our product candidates and could increase our costs of commercialization. Dependence on distribution arrangements and marketing alliances to commercialize our product candidates will subject us to a number of risks, including:

- we may not be able to control the amount and timing of resources that our distributors may devote to the commercialization of our product candidates;

- our distributors may experience financial difficulties;

- business combinations or significant changes in a distributor's business strategy may also adversely affect a distributor's willingness or ability to complete its obligations under any arrangement; and

- these arrangements are often terminated or allowed to expire, which could interrupt the marketing and sales of a product and decrease our revenue.

If our third-party service providers are unable to perform in accordance with the terms of our agreements, our potential to generate future revenue from our product candidates would be significantly reduced and our business would be materially and adversely harmed.

We rely on other third parties to store and distribute drug supplies for our preclinical development activities and our clinical trials. Any performance failure on the part of our existing or future distributors could delay clinical development or regulatory approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

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Extensions, delays, suspensions or terminations of our preclinical development activities and our clinical trials as a result of the performance of our independent clinical investigators and contract research organizations will delay, and make more costly, regulatory approval for any product candidates that we may develop. Any change in a contract research organization during an ongoing preclinical development activity or clinical trial could seriously delay that trial and potentially compromise the results of the activity or trial.

We may not be successful in maintaining or establishing collaborations, which could adversely affect our ability to develop and, particularly in international markets, commercialize products.

For each of our product candidates, we are collaborating with physicians, patient advocacy groups, foundations and government agencies in order to assist with the marketing and development of our products. We plan to pursue similar activities in future programs and plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies. We also may seek to establish collaborations for the sales, marketing and distribution of our products outside the United States. If we elect to seek collaborators in the future but are unable to reach agreements with suitable collaborators, we may fail to meet our business objectives for the affected product or program. We face, and will continue to face, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, document and implement. We may not be successful in our efforts, if any, to establish and implement collaborations or other alternative arrangements. The terms of any collaborations or other arrangements that we establish, if any, may not be favorable to us.

Any collaboration that we enter into may not be successful. The success of our collaboration arrangements, if any, will depend heavily on the efforts and activities of our collaborators. It is likely that any collaborators of ours will have significant discretion in determining the efforts and resources that they will apply to these collaborations. The risks that we may be subject to in possible future collaborations include the following:

- our collaboration agreements are likely to be for fixed terms and subject to termination by our collaborators in the event of a material breach or lack of scientific progress by us;

- our collaborators are likely to have the first right to maintain or defend our intellectual property rights and, although we would likely have the right to assume the maintenance and defense of our intellectual property rights if our collaborators do not, our ability to do so may be compromised by our collaborators' acts or omissions; and

- our collaborators may utilize our intellectual property rights in such a way as to invite litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential liability.

Furthermore, collaborations with pharmaceutical companies and other third parties often are terminated or allowed to expire by the other party. Such terminations or expirations may adversely affect us financially and could harm our business reputation in the event we elect to pursue collaborations that ultimately expire or are terminated.

Our future success depends on our ability to retain our chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on principal members of our management team and scientific staff. These executives each have significant pharmaceutical industry experience, including Horacio Plotkin, our Chief Medical Officer, Marc Panoff, our Chief Financial Officer, and one of our Directors. In addition, Martin Shkreli, our Chief Executive Officer, has significant experience investing in biopharmaceutical companies. We do not maintain "key person" insurance on Mr. Shkreli or on any of our other executive officers. We currently have employment agreements with our Chief Executive Officer, Chief Medical Officer and Chief Financial Officer.

Recruiting and retaining qualified scientific personnel, clinical personnel and sales and marketing personnel will also be critical to our success. Our industry has experienced a high rate of turnover in recent years. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. Although we believe we offer competitive salaries and benefits, we may have to increase spending in order to retain personnel.

We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

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We expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We are a development stage company with 26 full-time employees and five consultants. Of these employees and consultants, ten work primarily in research and development and five provide administrative services. We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development and regulatory affairs. 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.

In the event that we attempt to acquire or develop our own in-house sales, marketing and distribution capabilities, 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 creating our own sales and marketing team or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

Risks Related to the Development and Commercialization of Our Product Candidates

We face substantial risks related to the development and 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, Syntocinon, RE-034, RE-024, sparsentan and RE-001. 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 Syntocinon, RE-034, RE-024, sparsentan and RE-001, 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 payors;

- 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.

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If the market opportunities for our 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 IS, NS, PKAN, FSGS and DMD, 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 IS, NS, PKAN, FSGS and DMD, and 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 IS, NS, PKAN, FSGS or DMD in the study populations accurately reflect the prevalence of these diseases in the broader world population. If our estimates of the prevalence of IS, NS, PKAN, FSGS or DMD or of the number of patients who may benefit from treatment with RE-034, RE-024, sparsentan or RE-001 prove to be incorrect, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

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

Even if we are able to obtain and maintain regulatory approvals for our new pharmaceutical products, generic or branded, the success of these 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.

Any products that we bring to the market, including Syntocinon, RE-034, RE-024, sparsentan and RE-001—if they receive marketing approval—may not gain market acceptance by physicians, patients, third-party payors, 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 payors 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.

Initial results from pre-clinical and clinical studies do not ensure that future clinical trials will be successful.

We will only obtain regulatory approval to commercialize product candidates if we can demonstrate to the satisfaction of the FDA, or applicable non-United States 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

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indication. Clinical trials can be lengthy, complex and extremely expensive processes with uncertain results. A failure of one or more of our clinical trials may occur at any stage of testing. We have limited experience in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA.

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. For example, we have not identified a lead molecule in our RE-024 series of compounds, and we cannot be certain that a candidate suitable for a clinical study will ever be identified. Further, we have not begun pre-clinical evaluation of RE-001, and rely on external pre-clinical data for a closely related molecule. We cannot assure you that the pre-clinical data generated to date on TAT-u-UTR, a fusion protein between microutrophin and the TAT sequence from human immunodeficiency virus ("HIV") which is expected to transport molecules into cells for the treatment of muscular dystrophies, including DMD, will be representative of data for RE-001. We cannot assure you that any future clinical trials of Syntocinon, RE-034, RE-024, sparsentan or RE-001 will ultimately be successful.

Patients may not be compliant with their dosing regimen or trial protocols or they may withdraw from the study at any time for any reason. Even if our early-stage clinical trials are successful, we will need to conduct additional clinical trials with larger numbers of patients receiving the drug for longer periods for all of our product candidates before we are able to seek approvals to market and sell these product candidates from the FDA and regulatory authorities outside the United States. To date, we are not aware of any product to treat PKAN, FSGS or DMD that has been approved by the FDA. As a result, we cannot be sure what endpoints the FDA will require us to measure in later-stage clinical trials of our product candidates. If we are not successful in commercializing any of our development-stage products, or are significantly delayed in doing so, our business may be materially harmed.

We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA.

We have limited experience in conducting and managing the preclinical development activities and clinical trials necessary to obtain regulatory approvals, including approval by the FDA. We have not obtained regulatory approval or commercialized any product candidates. We are currently planning pre-clinical and eventual clinical studies for Syntocinon, RE-034, RE-024, sparsentan and RE-001. We plan to file a request for reactivation with the FDA with respect to Syntocinon in the first quarter of 2014. Approval of the NDA for Syntocinon was withdrawn by the FDA after Novartis withdrew the product from the market for commercial reasons. Accordingly, an approved NDA will need to be in place before we can reintroduce the product to the market. The FDA could request that we submit and obtain approval of a new NDA before the product can be reintroduced. We plan to re-launch Syntocinon in the first half of 2014. However, we may be required to submit additional information to the FDA and approval, if at all, may be delayed.

We filed an IND for the FSGS indication on July 2, 2012 and have received FDA clearance to begin a clinical study of sparsentan in FSGS, but have not filed INDs for RE-024 or RE-001. Although we plan to file an IND for the IS and NS designations for RE-034 in 2014, we cannot be certain that we will ever file INDs for any of RE-034, RE-024 or RE-001. Our limited experience might prevent us from successfully designing or implementing any clinical trials. We have limited experience in conducting and managing the application process necessary to obtain regulatory approvals and we might not be able to demonstrate that our product candidates meet the appropriate standards for regulatory approval. If we are not successful in conducting and managing our pre-clinical development activities or clinical trials or obtaining regulatory approvals, we might not be able to commercialize our developmental product candidates, or might be significantly delayed in doing so, which may materially harm our business.

We may find it difficult to enroll patients in our clinical trials for product candidates addressing rare diseases.

Certain of our product candidates that intended to treat IS, NS, PKAN, FSGS and DMD, 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 other non-United States regulatory agencies. 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.

If our preclinical studies do not produce positive results, if our clinical trials are delayed, or if serious side effects are identified during drug development, we may experience delays, incur additional costs and ultimately be unable to commercialize our product candidates.

Before obtaining regulatory approval for the sale of our product candidates, we must conduct, at our own expense, extensive preclinical and clinical tests to demonstrate the safety of our product candidates in animals and in humans. Preclinical and

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clinical 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 or clinical trials can occur at any stage of testing. 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.

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

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials. We will face an even greater risk if we obtain new products for sale or win approval for any of our drugs in development. 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;

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- 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.

Our business activities involve the use of hazardous materials, which require compliance with environmental and occupational safety laws regulating the use of such materials. If we violate these laws, we could be subject to significant fines, liabilities or other adverse consequences.

Our research and development programs involve the controlled use of hazardous materials, including microbial agents, corrosive, explosive and flammable chemicals and other hazardous compounds in addition to certain biological hazardous waste. Ultimately, the activities of our third-party product manufacturers when a product candidate reaches commercialization will also require the use of hazardous materials. Accordingly, we are subject to

federal, state and local laws governing the use, handling and disposal of these materials. Although we believe that our safety procedures for handling and disposing of these materials comply in all material respects with the standards prescribed by local, state and federal regulations, we cannot completely eliminate the risk of accidental contamination or injury from these materials. In addition, our collaborators may not comply with these laws. In the event of an accident or failure to comply with environmental laws, we could be held liable for damages that result, and any such liability could exceed our assets and resources or we could be subject to limitations or stoppages related to our use of these materials which may lead to an interruption of our business operations or those of our third-party contractors. While we believe that our existing insurance coverage is generally adequate for our normal handling of these hazardous materials, it may not be sufficient to cover pollution conditions or other extraordinary or unanticipated events. Furthermore, an accident could damage or force us to shut down our operations. Changes in environmental laws may impose costly compliance requirements on us or otherwise subject us to future liabilities and additional laws relating to the management, handling, generation, manufacture, transportation, storage, use and disposal of materials used in or generated by the manufacture of our products or related to our clinical trials. In addition, we cannot predict the effect that these potential requirements may have on us, our suppliers and contractors or our customers.

We may be unable to identify, acquire, close or integrate acquisition targets successfully.

Part of our business strategy includes acquiring and integrating complementary businesses, products, technologies or other assets, and forming strategic alliances, joint ventures and other business combinations, to help drive future growth. We may also in-license new products or compounds. Acquisitions or similar arrangements may be complex, time consuming and expensive. We may not consummate some negotiations for acquisitions or other arrangements, which could result in significant diversion of management and other employee time, as well as substantial out-of-pocket costs. In addition, there are a number of risks and uncertainties relating to our closing transactions. If such transactions are not completed for any reason, we will be subject to several risks, including the following: (i) the market price of our common shares may reflect a market assumption that such transactions will occur, and a failure to complete such transactions could result in a negative perception by the market of us generally and a decline in the market price of our common shares; and (ii) many costs relating to such transactions may be payable by us whether or not such transactions are completed.

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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 acquisition or arrangement after we have expended resources on them.

Risks Related to Regulatory Approval of Our Product Candidates

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found to have improperly promoted off-label uses, we may become subject to significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, such as our product candidates. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. The FDA may impose further requirements or restrictions on the distribution or use of our product candidates as part of a Risk Evaluation Mitigation Strategies, or REMS, plan, such as limiting prescribing to certain physicians or medical centers that have undergone specialized training, limiting treatment to patients who meet certain safe-use criteria and requiring treated patients to enroll in a registry. If we receive marketing approval for our product candidates, physicians may nevertheless prescribe our products to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

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

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

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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 (as further discussed below), among other things, amends the intent requirement of the federal anti-kickback statute. 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 false claims statutes. Although there are a number of statutory exemptions and regulatory safe harbors under the federal anti-kickback statute protecting certain common manufacturer business arrangements and activities from prosecution, the exemptions 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 exemptions and safe harbors whenever possible, but our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability and may be subject to scrutiny. Violations of the federal anti-kickback statute can result in civil and criminal fines and penalties and related administrative sanctions, including exclusion from federal health care programs.

The Federal False Claims Act prohibits any person 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 laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, which apply regardless of the payor. 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, California, Connecticut, Nevada, and Massachusetts require pharmaceutical companies to implement compliance programs or marketing codes.

Compliance with various federal and state laws is difficult and time consuming, and companies that violate them may face substantial penalties. The potential sanctions include civil monetary penalties, exclusion from participation in federal health care programs, criminal fines and imprisonment. Because of the breadth of these laws and the lack of extensive legal guidance in the form of regulations or court decisions, 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.

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.

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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 False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations and, beginning in March 2014 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 what 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.

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.

If we are not able to obtain and maintain required regulatory approvals, we will not be able to commercialize our product candidates, 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-party contract research organizations to assist us in these processes. If our third-party contract research organizations fail to adequately adhere to the regulation on drug sales 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 not obtained regulatory approval to market any of our product candidates in any jurisdiction. We have only limited experience in filing and prosecuting the applications necessary to obtain regulatory approvals and expect to rely on third-party contract research organizations to assist us in this process.

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

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

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

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

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

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

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

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

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

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

Our 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. We expect to seek orphan drug designations from the FDA for RE-034, RE-024, sparsentan and RE-001 though there can be no assurance that the FDA will grant orphan status. We also expect to seek drug orphan designation from the European Medicines Agency (the "EMA"), for RE-034, RE-024, sparsentan and RE-001. There can be no assurance that we will successfully obtain such designation. 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 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, sparsentan and RE-001 may be important to the product candidate's success. Even if we obtain orphan drug exclusivity for RE-034, RE-024 for PKAN, sparsentan for FSGS and RE-001 for DMD, 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

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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.

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;
- 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 payors 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 Syntocinon, RE-034, RE-024, sparsentan, RE-001 or any other product candidate that we develop, restrict or regulate post-approval activities and affect our ability to profitably sell Syntocinon, RE-034, RE-024, sparsentan, RE-001 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.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (the “MMA”), changed the way Medicare covers and pays for pharmaceutical products. As a result of this legislation and the expansion of federal coverage of drug products, Retrophin expects that there will be additional pressure to contain and reduce costs. These cost

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reduction initiatives and other provisions of this legislation could decrease the coverage and price that is received for any approved products and could seriously harm our business. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates, and any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

More recently, in March 2010, President Obama signed into law the Patient Protection and Affordable Care Act and the associated reconciliation bill (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. Effective October 1, 2010, the Health Care Reform Law revises the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states once the provision is effective. Further, beginning in 2011, the new law imposes a significant annual fee on companies that manufacture or import branded prescription drug products. The full effects of the Health Care Reform Law will not be known until applicable federal and state agencies issue regulations or guidance under the new law. Although it is too early to determine the effect of the Health Care Reform Law, the new 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 adequate reimbursement from governments or third-party payors 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 adequate reimbursement for the use of our approved product candidates from governmental and other third-party payors, both in the United States and in other markets. Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor'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 payor 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 payor. 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 payors' satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources. Even when a payor determines that a product is eligible for

reimbursement, the payor 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. For example, the MMA provides a new Medicare prescription drug benefit that began in 2006 and mandates other reforms. While we cannot predict the full outcome of the implementation of this legislation, it is possible that the new Medicare prescription drug benefit, which will be managed by private health insurers and other managed care organizations, will result in additional government reimbursement for prescription drugs, which may make some prescription drugs more affordable but may further exacerbate industry-wide pressure to reduce prescription drug prices. If one or more of our product candidates reaches commercialization, such changes may have a significant impact on our ability to set a price we believe is fair for our products and may affect our ability to generate revenue and achieve or maintain profitability.

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

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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 Common Stock

Our executive officers, directors and principal stockholders have the ability to strongly influence all matters submitted to our stockholders for approval.

Martin Shkreli, our Chief Executive Officer and one of our directors, is our largest stockholder. Together with other entities that he controls, Mr. Shkreli beneficially owns 3,445,615 shares of our common stock, or approximately 19% of our outstanding common stock. If he were to choose to act with other large stockholders, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, will control the election of directors and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders, and they may act, whether by meeting or written consent of stockholders, in a manner that advances their best interests and not necessarily those of other stockholders, including obtaining a premium value for their common stock, and might affect the prevailing market price for our common stock.

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;
- 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 securities market has from time to time experienced significant price and volume fluctuations that are not related to the operating performance of particular companies. These market fluctuations may also materially and adversely affect the market price of shares of our common stock.

For these reasons and others you should consider an investment in our common stock as risky and invest only if you can withstand a significant loss and wide fluctuations in the market value of your investment.

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We do not anticipate paying cash dividends in the foreseeable future and, as a result, our investors' sole source of gain, if any, will depend on capital appreciation, if any.

We have never paid cash dividends on our capital stock and we do not anticipate paying any cash dividends in the foreseeable future. You should not invest in us if you require dividend income. Any income from an investment in us would only come from a rise in the market price of our common stock, which is uncertain and unpredictable.

We have paid no cash dividends on our capital stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business and do not foresee payment of a dividend in any upcoming fiscal period. In addition, the terms of existing or any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

If securities or industry analysts do not publish research or reports or publish unfavorable research about our business, the price of our common stock and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. We do not currently have and may never obtain research coverage by securities and industry analysts. If no securities or industry analysts commence coverage of us, the trading price for our common stock would be negatively affected. In the event we obtain securities or industry analyst coverage, if one or more of the analysts who covers us downgrades our common stock, the price of our common stock would likely decline. If one or more of these analysts ceases to cover us or fails to publish regular reports on us, interest in the purchase of our common stock could decrease, which could cause the price of our common stock or trading volume to decline.

If we fail to comply with the rules and regulations under the Sarbanes-Oxley Act, our operating results, our ability to operate our business and investors' views of us may be harmed.

We are required to comply with the rules and regulations under the Sarbanes-Oxley Act. Section 404 of the Sarbanes-Oxley Act requires public companies to conduct an annual review and evaluation of their internal controls and attestations of the effectiveness of internal controls by independent auditors. Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that will need to be evaluated frequently. Our failure to maintain the effectiveness of our internal controls in accordance with the requirements of the Sarbanes-Oxley Act could have a material adverse effect on our business. We could lose investor confidence in the accuracy and completeness of our financial reports, which could have an adverse effect on the price of our common stock.

In addition, our efforts to comply with the rules and regulations under the Sarbanes-Oxley or new or changed laws, regulations, and standards may differ from the activities intended by regulatory or governing bodies due to ambiguities related to practice. Regulatory authorities may investigate transactions disclosed in our "Management's Discussion and Analysis of Financial Condition and Results of Operations", and if legal proceedings are initiated against us, it may harm our business.

Provisions in our bylaws could discourage, delay or prevent a change of control of our company and may result in an entrenchment of management and diminish the value of our common stock.

Our bylaws provide that, unless otherwise prescribed by statute or the certificate of incorporation, special meetings of the stockholders can only be called by our President, by a majority of the Board of Directors, or at the written request of stockholders owning at least 50% in amount of the entire capital stock of the Company issued and outstanding and entitled to vote. These provisions may discourage, delay or prevent a merger, acquisition or other change of control that our stockholders may consider favorable. Such provisions could impede the ability of our common stockholders to benefit from a change of control and, as a result, could materially adversely affect the market price of our common stock and your ability to realize any potential change-in-control premium.

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CAUTIONARY NOTE ON FORWARD LOOKING STATEMENTS

Certain information contained in this prospectus include forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The statements herein which are not historical reflect our current expectations and projections about the our future results, performance, liquidity, financial condition, prospects and opportunities and are based upon information currently available to us and our management and their interpretation of what is believed to be significant factors affecting the businesses, including many assumptions regarding future events. Such forward-looking statements include statements regarding, among other things:

- our ability to obtain regulatory approval for, and produce, market and generate sales of, our products;
- commencement and completion of clinical trials of our products;
- our projected future sales, profitability and other financial metrics;
- our future financing plans;
- our plans for expansion of our facilities;
- our anticipated needs for working capital;
- the anticipated trends in our industry;
- our ability to expand our sales and marketing capability;
- acquisitions of other companies or assets that we might undertake in the future;
- our operations in the United States and abroad, and the domestic and foreign regulatory, economic and political conditions; and

- competition existing today or that will likely arise in the future.

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

Potential investors should not place undue reliance on any forward-looking statements. Except as expressly required by the federal securities laws, there is no undertaking to publicly update or revise any forward-looking statements, whether as a result of new information, future events, changed circumstances or any other reason.

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

Potential investors should not make an investment decision based solely on our projections, estimates or expectations.

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USE OF PROCEEDS

We estimate that the net proceeds from our sale of 4,705,882 shares of our common stock in this offering at a public offering price of $8.50 per share, after deducting underwriting discounts and commissions and estimated offering expenses, will be approximately $36.6 million, or $42.2 million if the underwriters' option to purchase additional shares is exercised in full.

The principal purposes of this offering are to obtain additional capital to support our operations, to create a public market for our common stock on a national securities exchange and to facilitate our future access to the public equity markets. We anticipate that we will use the net proceeds of this offering as follows:

- approximately $7 million to obtain regulatory approval for the reintroduction of Syntocinon into the US market for aiding milk letdown, and to initiate a sales and education campaign on the benefits of Syntocinon in the treatment of milk letdown;
- approximately $2 million to initiate Phase 2 clinical trials of Syntocinon for the treatment of Schizophrenia;
- approximately $4 million to initiate Phase 2 clinical trials of Syntocinon for the treatment of Autism Spectrum Disorders;
- approximately $2 million to fund initial clinical development of RE-034 through a planned Phase 3 clinical trial for the treatment of IS;
- approximately $3 million to fund initial clinical development of RE-034 through a planned Phase 3 clinical trial for the treatment of NS;
- approximately $2 million to initiate a safety study and fund initial clinical development of RE-024 for the treatment of PKAN; and
- approximately $5 million to complete Phase 2 trial of sparsentan for the treatment of FSGS.

We will use the remaining net proceeds, if any, for the further advancement of our early-stage development programs; for further product development; and for general corporate purposes, such as general and administrative expenses, working capital, and prosecution and maintenance of our intellectual property rights. This offering is also intended to facilitate our future access to public markets. We reserve the right to change the use of these proceeds as a result of certain contingencies such as competitive developments, the results of our commercialization efforts, acquisition and investment opportunities and other factors. We believe that our existing cash and marketable securities as of the date of this offering, together with the proceeds of this offering, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements for the next 12 months.

We may also use a portion of the net proceeds from this offering to in-license, acquire, or invest in complementary businesses, technologies, products or assets. We may also invest in public equity securities. However we have no current plan, commitments or obligations to do so.

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual use of net proceeds will vary depending on numerous factors, including the factors described under “Risk Factors” in this prospectus. As a result, management will have broad discretion in the application of the net proceeds, and investors will be relying on our judgment regarding the application of the net proceeds of this offering. Even with the expected net proceeds from this offering, we do not expect to have sufficient cash to complete the clinical development of any of our product candidates.

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DIVIDEND POLICY

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business and do not intend to declare or pay any cash dividends in the foreseeable future. As a result, you will likely need to sell your shares of common stock to realize a return on your investment, and you may not be able to sell your shares at or above the price you paid for them. Payment of cash dividends, if any, in the future will be at the discretion of our board of directors and will depend on then-existing conditions, including our financial condition, operating results, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

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CAPITALIZATION

The following table sets forth our capitalization as of September 30, 2013:

- on an actual basis; and
- on an as adjusted basis to give effect to the sale of the 4,705,882 shares of our common stock that we are offering at a public offering price of $8.50 per share after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this table with our financial statements and related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations" incorporated by reference in this prospectus.

	As of September 30, 2013	
	Actual	As Adjusted
	(Unaudited)	
Cash, marketable securities, available for sale and prepaid expenses and other current assets	$16,847,848	$ 53,447,848
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value, 20,000,000 Shares authorized; 0 issued and outstanding	—	—
Common stock, $0.0001 par value: 100,000,000 shares authorized; 18,376,363 shares issued and outstanding, actual; 23,082,245 shares issued and outstanding, as adjusted	1,838	2,309
Additional paid-in capital	48,649,970	85,249,499
Deficit accumulated during the development stage	(54,301,348)	(54,301,348)
Accumulated other comprehensive income	(154,834)	(154,834)
Total stockholders' equity (deficit)	(5,804,374)	30,795,626
Total capitalization	$(5,804,374)	$ 30,795,626

The number of shares of common stock shown above is based on 18,376,363 shares of common stock outstanding as of September 30, 2013. This number excludes:

- 260,000 shares of common stock issuable upon the exercise of options outstanding as of September 30, 2013, at a weighted-average exercise price of $7.12 per share; and
- 4,462,426 shares of our common stock issuable upon the exercise of warrants outstanding as of September 30, 2013 at a weighted-average exercise price of $5.14 per share.

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DILUTION

If you invest in our common stock, you will experience immediate and substantial dilution to the extent of the difference between the public offering price of our common stock in this offering and the as adjusted net tangible book value per share of our common stock immediately after the offering.

Our historical net tangible book value (deficit) per share is determined by dividing our total tangible assets, less total liabilities, by the actual number of outstanding shares of our common stock. The historical net tangible book value (deficit) of our common stock as of September 30, 2013 was $(7.9) million, or $(0.43) per share.

After giving effect to the sale of 4,705,882 shares of our common stock offered by us at a public offering price of $8.50 per share, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, our net tangible book value as of September 30, 2013 would have been approximately $28.7 million, or $1.25 per share of common stock. This represents an immediate increase in net tangible book value of $1.68 per share to existing stockholders and an immediate dilution of $7.25 per share to new investors purchasing shares of common stock in this offering at the assumed public offering price. The following table illustrates this dilution on a per share basis:

Public offering price per share		$8.50
Net tangible book value (deficit) per share as of September 30, 2013	$(0.43)	
Increase per share attributable to investors purchasing our common stock in this offering	1.68	
As adjusted net tangible book value per share after this offering		1.25
Dilution per share to investors purchasing our common stock in this offering		$7.25

If the underwriters exercise in full their option to purchase up to 705,882 of additional shares of common stock at the public offering price of $8.50 per share, the as adjusted net tangible book value after this offering would be $1.44 per share, representing an increase in net tangible book value of $1.87 per share to existing stockholders and immediate dilution in net tangible book value of $7.06 per share to investors purchasing our common stock in this offering at the assumed public offering price.

The above discussion and table are based on 18,376,363 shares of common stock outstanding as of September 30, 2013 and excludes:

- 260,000 shares of common stock issuable upon the exercise of options outstanding as of September 30, 2013, at a weighted-average exercise price of $7.12 per share; and

- 4,462,426 shares of our common stock issuable upon the exercise of warrants outstanding as of September 30, 2013 at a weighted-average exercise price of $5.14 per share.

To the extent that options or warrants outstanding as of September 30, 2013 have been or are exercised, investors purchasing shares in this offering could experience further dilution.

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Price range of common stock

Prior to this offering, our common stock was listed for quotation on the OTC QB market under the trading symbol "RTRX" ("DGTE" prior to December 17, 2012). There was limited trading in our shares since they became eligible for trading on the OTC QB market during the third quarter of 2008. In connection with this offering, our common stock has been approved for listing on The NASDAQ Global Market under the symbol "RTRX.".

The following table sets forth the high and low bid prices for our common stock for the periods indicated as reported by the OTC QB ("N/A" indicates no trading during such period). The below quotations reflect inter-dealer prices, without retail mark-up, mark-down or commission, and may not represent actual transactions.

Quarter Ending	High	Low
Fiscal Year 2014		
First Quarter (through January 9, 2014)	$8.58	$7.19
Fiscal Year 2013		
First Quarter	$5.78	$3.00
Second Quarter	$9.99	$4.75
Third Quarter	$7.25	$4.50
Fourth Quarter	$9.00	$5.25
Fiscal Year 2012		
First Quarter	N/A	N/A
Second Quarter	$1.05	$1.05
Third Quarter	$1.05	$1.05
Fourth Quarter	$3.00	$0.13
Fiscal Year 2011		
First Quarter	$0.90	$0.90
Second Quarter	$0.90	$0.90
Third Quarter	$1.05	$0.90
Fourth Quarter	$1.05	$0.90
Fiscal Year 2010		
First Quarter	N/A	N/A
Second Quarter	$0.90	$0.90
Third Quarter	$0.90	$0.90
Fourth Quarter	$0.90	$0.90

As of January 9, 2014, we had approximately 290 holders of record of our common stock.

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MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion includes forward-looking statements about our business, financial condition and results of operations, including discussions about management's expectations for our business. These statements represent projections, beliefs and expectations based on current circumstances and conditions and in light of recent events and trends, and you should not construe these statements either as assurances of performance or as promises of a given course of action. Instead, various known and unknown factors are likely to cause our actual performance and management's actions to vary, and the results of these variances may be both material and adverse. A description of material factors known to us that may cause our results to vary, or may cause management to deviate from its current plans and expectations, is set forth under "Risk Factors." See "Cautionary Note Regarding Forward-Looking Statements." The following discussion should also be read in conjunction with our audited and unaudited consolidated financial statements, including the notes thereto, and unaudited pro forma combined financial statements appearing elsewhere in this prospectus.

Overview

We are a development-stage biopharmaceutical company focused on the development, acquisition and commercialization of therapies for the treatment of serious, catastrophic or rare diseases. We are developing Syntocinon TM Nasal Spray in the U.S. to assist initial postpartum milk ejection, and for the treatment of Schizophrenia and Autism. Syntocinon Nasal Spray is currently marketed by Novartis and Sigma-Tau in Europe and other countries for aiding milk let-down. In addition, we are developing RE-034, a synthetic hormone analogue that is composed of the first 24 amino acids of the 39 amino acids contained in ACTH for the treatment of IS and NS. We are developing RE-024, a novel small molecule, as a potential treatment for PKAN. Also, we are developing sparsentan, formerly known as RE-021, a dual acting receptor antagonist of angiotensin and endothelin receptors, for the treatment of FSGS. We also have several additional programs in preclinical development, including RE-001, a therapy for the treatment of DMD.

Our results of operations discussed below reflect our operations during the period in which we are in development stage and starting up our operations. As a result, these results should not be considered indicative of our anticipated results of operations on a going forward basis.

Restatement of December 31, 2012 Financial Statements

On September 13, 2013, we determined that we were required to file an amendment to our audited consolidated financial statement for the year ended December 31, 2012 included in our Transitional Report on Form 10-K. We determined, after consultation with our board of directors and our independent registered public accounting firm that it would be necessary to restate our December 31, 2012 consolidated financial statements to include disclosures of certain agreements that we entered into subsequent to the date of the balance sheet and corrections to our accounting for proceeds received in a financing transaction we completed in February 2013. The addition of these footnote disclosures in our December 31, 2012 consolidated financial statements had no impact on our balance sheet, or related consolidated statements of operations, changes in stockholders' (deficit) equity, loss per share or cash flows for the year ended December 31, 2012. On September 16, 2013, we amended our Transition Report on Form 10-K for the transition period from March 1, 2012 to December 31, 2012, as filed with the SEC on June 13, 2013, solely for the purpose of amending Part II, Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations and Part II, Item 8. Financial Statements and Supplementary Data to include disclosure and a footnote to our financial statements relating to events that occurred after the conclusion of the period covered by the original filing. The disclosure below reflects the changes made in such amendment. We further determined that it would be necessary for us to restate our March 31, 2013 condensed consolidated financial statements to include the same disclosures in these financial statements that we were required to make in our December 31, 2012 financial statements, and to correct our accounting for the allocation of $360,000 in proceeds we received in the financing transaction we completed in February 2013.

Financial Overview

The following discussion summarizes the key factors our management believes are necessary for an understanding of our financial statements.

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Compensation and Related Costs

Our compensation and related costs consist primarily of salaries, benefits and stock-based compensation. We expect our compensation and related costs to increase as we expand our research and development programs and general and administrative activities.

Professional Fees

Professional fees consist of expenses for outside professional services, including legal, human resource, audit, tax and accounting services.

Research and Development

Research and development expenses represent costs incurred to conduct research of our proprietary product candidates. We expense all research and development costs as they are incurred. Our research and development expenses consist of employee salaries and related benefits, including stock-based compensation, third-party contract costs relating to research, manufacturing, preclinical studies, clinical trial activities, laboratory consumables, and allocated facility costs.

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

We routinely engage vendors and service providers for scientific research, clinical trial, regulatory compliance, manufacturing and other consulting services. To date, such engagements have been generally based on pre-determined prices or rates. We also make grants to research and non-profit organizations to conduct research which may lead to new intellectual properties that we may subsequently license under separately negotiated license agreements. Such grants may be funded in lump sums or installments.

The following table summarizes our research and development expenses during the years ended December 31, 2011 and 2012 and the nine months ended September 30, 2012 and 2013. The internal costs include personnel, facility costs, laboratory consumables and discovery and research related activities associated with our pipeline. The external program costs reflect external costs attributable to our clinical development candidates and preclinical candidates selected for further development. Such expenses include third-party contract costs relating to manufacturing, clinical trial activities, translational medicine and toxicology activities.

	Nine months ended September 30,		**Year ended December 31,**	**For the period from March 11, 2011 (inception) through December 31, 2011**
	2013	**2012**	**2012**	
External service provider costs:				
Sparsentan	$877,298	$ 109,205	$176,450	$—
RE-024	390,477	45,568	124,635	—
General	215,681	132,116	240,034	94,454
Other product candidates	144,528	—	—	258,940
Total external service providers costs:	1,627,984	286,889	541,119	353,394
Internal personnel costs:	485,829	—	—	—
Total research and development	$2,113,813	$ 286,889	$541,119	$353,394

We expect our research and development expenses will increase in the future as we progress our product candidates, advance our discovery research projects into the preclinical stage and continue our early stage research. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time consuming.

We may never succeed in achieving marketing approval for any of our product candidates. The probability of success of each product candidate may be affected by numerous factors, including preclinical data, clinical data, competition, manufacturing capability and commercial viability.

Most of our product development programs are at an early stage; therefore, the successful development of our product candidates is highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each product candidate and are difficult to predict. Given the uncertainty associated with clinical trial

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enrollments and the risks inherent in the development process, we are unable to determine the duration and completion costs of current or future clinical trials of our product candidates or if and to what extent we will generate revenues from the commercialization and sale of any of our product candidates. We anticipate that we and our strategic alliance partners will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to the scientific and clinical success of each product candidate, as well as an ongoing assessment as to each product candidate's commercial potential. We will need to raise additional capital or may seek additional strategic alliances in the future in order to complete the development and commercialization of our product candidates.

Selling, General and Administrative

Selling, general and administrative expenses consist of rent, depreciation and amortization, settlement charges, travel and entertainment, recruiting, insurance, business development, advertising, and other operating expenses. We expect to incur additional expenses as a result of operating as a public company, including costs to comply with the rules and regulations applicable to companies listed on a national securities exchange and costs related to compliance and reporting obligations pursuant to the rules and regulations of the Securities and Exchange Commission, or SEC. In addition, as a public company, we expect to incur increased expenses related to additional insurance, investor relations and other increases related to needs for additional human resources and professional services.

Other Expenses

Other expenses consist of charges from the change in fair value of derivative financial instruments, interest income and expense, charges from transactions denominated in foreign currencies, realized gains and losses on the sale of marketable securities, and registration payment income and expense.

License Agreements

Novartis

On December 12, 2013, we entered into an agreement with Novartis and Novartis AG pursuant to which Novartis and Novartis AG agreed to grant us an exclusive, perpetual, and royalty-bearing license for the manufacture, development and commercialization of Syntocinon and related intranasal products in the United States. Under the license, Novartis and Novartis AG are obligated to transfer to us certain information that is necessary for or related to the development or commercialization of Syntocinon. We are responsible for conducting research and preclinical, clinical and other development of Syntocinon at our own expense, and must use commercially reasonably efforts to develop Syntocinon in the United States.

As consideration for the license, we paid to Novartis and Novartis AG a $5 million upfront fee and are required to make substantial payments upon the achievement of certain milestones. Should we commercialize Syntocinon, we will be obligated to pay Novartis and Novartis AG a 20% royalty on net sales of such products. We are also required to pay annual maintenance fees to Novartis and Novartis AG. The license agreement contains other customary clauses and terms as are common in similar agreements in the industry.

Ligand

In February 2012, we entered into an agreement pursuant to which Ligand agreed to grant us a worldwide license for the development, manufacture and commercialization of sparsentan, an ARB and ERA which we are initially using in connection with the treatment of FSGS. Under the license agreement, Ligand granted us a sublicense under certain of its patents and other intellectual property in connection with the development and commercialization of sparsentan. Under the license agreement, Ligand is obligated to transfer to us certain information, records, regulatory filings, materials and inventory controlled by Ligand and relating to or useful for developing sparsentan. We must use commercially reasonable efforts to develop and commercialize sparsentan in specified major market countries and other countries in which we believe it is commercially reasonable to develop and commercialize such products.

As consideration for the license, we are required to make substantial payments payable upon the achievement of certain milestones totaling up to $106.9 million. Should we commercialize sparsentan or any products containing any of these compounds, we will be obligated to pay to Ligand an escalating annual royalty between 10% and 20% of net sales of all such products. In the event that we desire to enter into a license arrangement with respect to any licensed compound under the license agreement, Bristol-Myers Squibb Company will have a right of first negotiation and Ligand will have a right of second negotiation with respect to any such license arrangement for a licensed compound. The license agreement contains other customary clauses and terms as are common in similar agreements in the

industry. Through September 30, 2013, we made payments to Ligand of $2.55 million under the license agreement.
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Central Nervous System License

On December 12, 2013, we entered into an agreement, which we refer to as the "Weg License Agreement," with Stuart Weg, MD, pursuant to which Dr. Weg agreed to grant us an exclusive worldwide license for the manufacture, development and distribution of products to be developed for the treatment of central nervous system disorders. As consideration for the license, we are required to pay Dr. Weg an upfront fee, which amount included a $250,000 payment prior to the execution of the Weg License Agreement, as well as certain maintenance and sublicensing fees. We are also obligated to pay Dr. Weg certain royalties on sales of FDA-approved products.

The Weg License Agreement contains other customary clauses and terms as are common in similar agreements in the industry.

The Weg License Agreement will continue in perpetuity unless terminated by us or by Dr. Weg. We may terminate the agreement at any time by giving written notice to Dr. Weg. Dr. Weg may terminate the agreement due to our uncured material breach of the agreement.

Results of Operations

Three month period ended September 30, 2013 compared to the three month period ended September 30, 2012

Revenue. We had no revenues during the three month period ended September 30, 2013 and 2012.

Operating Expenses. Our operating expenses for the three month period ended September 30, 2013 were $5.2 million compared to $8.5 million for the three month period ended September 30, 2012 which represents a decrease of $3.3 million, or 39%. The expense decrease was principally attributable to a decrease in our compensation and related costs in the amount of $4.6 million as well as a decrease in our professional fees in the amount of $0.7 million, offset by an increase in our research and development expenses in the amount of $1.3 million and an increase in our selling, general and administrative costs in the amount of $0.7 million. Included in the decrease in our compensation and related costs is a decrease in stock based compensation to employees of $4.8 million and an increase in cash compensation to employees of approximately $200,000 due to an increase in the number of employees. Included in the decrease in our professional fees is a decrease in stock based compensation to non-employees of $0.6 million and a decrease in cash compensation to professionals of approximately $100,000 due to a decrease in consulting fees. Included in selling, general and administrative costs are settlement charges in the amount of $300,000 and an increase in cash expenditures of approximately $300,000 due to an increase in travel and related expenses associated with business development.

Other Expenses. Other expense for the three month period ended September 30, 2013 was $5.7 million compared to other expense of $20,712 for the three month period ended September 30, 2012, which represents an increase of $5.7 million. The increase was primarily attributable to loss from the change in fair value of derivative financial instruments of $5.8 million a decrease in interest income of $6,049, offset by a realized gain on the sale of marketable securities of $59,737 and a decrease in interest expense of $26,761. Included in other income is registration payment income of $360,000 relating to a waiver we received for previous liquidated damages and expense of $360,000 from allocating the waiver of the original registration payment from the February 14, 2013 registration rights agreement as a charge to income.

Net Loss. Our net loss for the three month period ended September 30, 2013 was $10.9 million compared to $8.5 million for the three month period ended September 30, 2012.

Nine month period ended September 30, 2013 compared to the nine month period ended September 30, 2012

Revenue. We had no revenues during the nine month period ended September 30, 2013 and 2012.

Operating Expenses. Our operating expenses for the nine month period ended September 30, 2013 were $12.5 million compared to $16.8 million for the nine month period ended September 30, 2012 which represents a decrease of $4.3 million, or 26%. The expense decrease was primarily attributable to a decrease in our compensation and related costs in the amount of $6.6 million as well as a decrease in our professional fees in the amount of $3.4 million, offset by an increase in our research and development expenses in the amount of $1.8 million, an increase in our selling, general and administrative costs in the amount of $3.8 million and an increase in our technology license fee in the amount of $100,000. Included in the decrease in our compensation and related costs is a decrease in stock based compensation to employees of $7.7 million and an increase in cash compensation to employees of approximately $1 million due to an increase in the number of employees. Included in the decrease in our professional fees is a decrease in stock based compensation to non-employees of $4.4 million and an increase in cash compensation to professionals

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million due to an increase in consulting and legal fees associated with business development. Included in selling, general and administrative costs are settlement charges in the amount of $2.6 million and an increase in cash expenditures of approximately $1.2 million due to an increase in travel and related expenses associated with business development.

Other Expenses. Other expenses for the nine month period ended September 30, 2013 was $8.2 million compared to $54,778 for the nine month period ended September 30, 2012 which represents an increase of $8.1 million. The expense increase was primarily attributable to the expense from the change in fair value of derivative financial instruments of $8.2 million, a decrease in interest income of $15,772, an increase in loss on transactions denominated in foreign currencies of $3,873, offset by a realized gain on the sale of marketable securities of $59,737 and a decrease in interest expense of $28,996. Included in other income is registration payment income of $360,000 related to the waiver we received for previous liquidated damages and expense of $360,000 from allocating the waiver of the original registration payment from the February 14, 2013 registration rights agreement as a charge to income.

Net Loss. Our net loss for the nine month period ended September 30, 2013 was $20.7 million compared to $16.8 million for the nine month period ended September 30, 2012.

Year ended December 31, 2012

Revenue. We had no revenue during the year ended December 31, 2012

Operating Expenses. Operating expenses were approximately $30.26 million for the year ended December 31, 2012, which consisted of:

- compensation and related costs of approximately $18.13 million which included approximately 2,048,000 shares of vested incentive shares granted to members and employees amounting to approximately $16.01 million;

- professional fees of approximately $9.04 million which included

- approximately 194,000 shares of vested incentive shares granted to consultants and direct transfers of shares to consultants by members amounting to approximately $6.40 million for services rendered;

- research and development fees of approximately $0.52 million related to Retrophin’s drug (sparsentan and RE-024) candidate for the treatment of FSGS and PKAN and evaluation of potential new technologies;

- legal expense of approximately $0.91 million related to licensing and production acquisition, employment and consulting agreements and general corporate work;

- consulting fees of approximately $0.83 million related to outsourcing management roles;

- contracted services of approximately $0.11 million; and

- accounting fees of approximately $0.26 million related to general accounting and audit work, (iii) twelve months rent expense of approximately $0.1 million;

- license fee of approximately $1.70 million;

- depreciation and amortization expense of approximately $0.12 million related to the Ligand licensing agreement;

- had debt expense of $0.56 million; and

- the remaining balance of $0.61 million is related to travel and entertainment, advertising and other operating expenses.

Other Expenses. Other expenses for the year ended December 31, 2012 were as follows: (i) approximately $0.003 million, which is related to a loss in foreign exchange in a vendor payment, (ii) approximately $0.022 million, which related to $0.2 million note receivable with an interest rate of 12% per annum offset by approximately $0.106 million of interest expense relate to a $0.900 million and $0.030 note payable with an interest rate of 12% and 15%, respectively, per annum.

Net Loss. For the year ended December 31, 2012, our net loss from operations was approximately $30.26 million.

March 11, 2011 (inception) through December 31, 2011

Revenue. We had no revenue during the period from March 11, 2011 (inception) through December 31, 2011.

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Operating Expenses. Operating expenses were approximately $3.27 million for the period from March 11, 2011 through December 31, 2011, which consisted of:

- compensation and related costs of approximately $2.23 million which included approximately 431,000 shares of vested incentive shares granted to members and employees amounting to approximately $1.72 million;
- professional fees of approximately $0.91 million which included:
- approximately 60,000 shares of vested incentive shares granted to consultants amounting to approximately $0.26 million for services rendered;
- research and development fees of approximately $0.35 million related to Retrophin's drug (RE-001) candidate for the treatment of Duchenne Muscular Dystrophy;
- legal expense of approximately $0.10 million related to formation of the company, employment and consulting agreements and general corporate work; and
- consulting fees of approximately $0.20 million related to outsourcing management roles;
- nine months rent expense of approximately $0.06 million; and
- the remaining balance of $0.07 million is related to travel and entertainment, depreciation, advertising and other operating expenses.

Other Expenses. Other operating expenses for the period March 11, 2011 (inception) through December 31, 2011 were approximately $5,000, which is related to a loss in foreign exchange in a vendor payment.

Income Taxes. As a limited liability company, we were treated as a partnership for the purposes of U.S. federal and most applicable state and local income tax during the start-up period from March 11, 2011 through September 21, 2012. Accordingly, no provision has been made for U.S. federal and state income taxes in the accompanying financial statements for such period, since all items of income or loss were required to be reported on the income tax returns of the members, who are responsible for any taxes thereon.

Net Loss. For the period March 11, 2011 (inception) through December 31, 2011, our net loss was approximately $3.27 million.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements.

Liquidity and Capital Resources

Management believes that we will continue to incur losses for the foreseeable future. Therefore we will either need additional equity or debt financing, or 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.

Our continued operations will depend on whether we can successfully raise additional funds through equity and/or debt financing. Such additional funds may not become available on acceptable terms, if at all, and we cannot assure you that any additional funding we do obtain will be sufficient to meet our needs in the long term. Since inception, through September 30, 2013, we had raised approximately $40.2 million through capital contributions and notes payable from Retrophin shareholders and related parties.

Since inception through September 30, 2013, we have incurred a net loss of approximately $54 million, including stock-based compensation charge of approximately $26 million for the period from March 11, 2011 (inception) to September 30, 2013. At September 30, 2013, we had a working capital deficit of approximately $10 million; however, the working capital deficit includes a derivative liability of approximately $22.2 million for warrants issued in financing transactions. Our accumulated deficit amounted to $54,301,348 at September 30, 2013.

Since our inception in 2011, we have generated losses from operations and we anticipate that we will continue to generate losses from operations for the foreseeable future. As of September 30, 2013 and December 31, 2012, our stockholders' deficit was $5,804,374 and $3,407,815, respectively. Our net loss from operations for the period March 11, 2011 (inception) through December 31, 2011, for the year ended December 31, 2012 and for the period March 11, 2011 (inception) through December 31, 2012 were approximately $3.27 million, $30.26 million and $33.52 million, respectively. Our net loss for the nine month period ended September 30, 2013 was $20,689,236 compared to $16,812,669 for the nine month period ended September 30, 2012. Net cash used in operating activities were $0.79 million, $2.74 million and $3.52 million for the period March 11, 2011 (inception) through December 31, 2011, for the

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year ended December 31, 2012 and for the period March 11, 2011 (inception) through December 31, 2012, respectively. Net cash used in operating activities was $9,442,442 for the nine month period ended September 30, 2013 compared to $2,088,811 for the nine month period ended September 30, 2012. Operations since inception have been funded entirely with the proceeds from equity and debt financings. As of September 30, 2013, we had cash of $13,409,825. We will continue to fund operations from cash on hand 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 desired development. 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.

In January 2013, we sold common stock in certain private placement transactions for aggregate proceeds of $816,664. In February, 2013, we sold common stock and warrants in a private placement transaction for aggregate proceeds of $9,137,787. In August, 2013, we sold common stock and warrants in a private placement transaction for aggregate proceeds of $24,891,303.

In the second quarter of 2013, the Company, its Chief Executive Officer, MSMB CAPITAL MANAGEMENT, LP (“MSMB Capital LP”), a Delaware limited partnership, MSMB CAPITAL MANAGEMENT LLC (“MSMB Capital LLC”), a Delaware limited liability company, MSMB HEALTHCARE LP (“MSMB Healthcare”), a Delaware limited partnership, MSMB HEALTHCARE INVESTORS LLC (“MSMB Investors”), a Delaware limited liability company, MSMB HEALTHCARE MANAGEMENT LLC (“MSMB Management” and, together with MSMB Capital LP, MSMB Capital LLC, MSMB Healthcare and MSMB Investors, the “MSMB Entities”), a Delaware limited liability company became parties to a series of agreements to settle up to $2,284,511 of liabilities owed to certain investors in the MSMB Entities which had invested in the Company and objected to the number of shares of common stock in the Company that they received as a distribution from such funds. Because the Company was a party to these settlements, it applied the accounting guidance provided in ASU 2013-04 (“ASU 2013-04”). This guidance requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount that the entity has (a) contractually agreed to pay and (b) any additional amounts that the entity expects to pay on behalf of its co-obligors. Company management believes such liabilities are the obligation of the MSMB Entities and concurrent with the execution and payment of such settlement agreements, the Company entered into indemnification agreements and received promissory notes from the MSMB Entities, whereby the MSMB Entities jointly and severally agreed to pay the Company the principal amount of $2,284,511, plus interest at an annualized rate of 5% as reimbursement of payments that the Company made to settle a portion of the agreements. The Company paid $593,111 of these settlements in the second quarter on behalf of the MSMB Entities and had outstanding liabilities of $1,691,400 as of September 30, 2013, which the Company has paid. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the third quarter ended September 30, 2013. There is uncertainty as to whether the MSMB Entities will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

On August 29, 2013, the Company entered into and paid an additional settlement agreement for $300,000 due following execution of the agreement.

Sponsored Research Agreements

St. Jude Sponsored Research Agreement

Effective October 1, 2013, we entered into the SRA with St. Jude, pursuant to which St. Jude will undertake a research program with respect to RE-024. As consideration for the research program, we are obligated to pay an aggregate of $780,674 in fees to St. Jude on a specified timeline, of which $195,168 has been paid as of the date hereof. Pursuant to the SRA, we granted St. Jude a non-exclusive, royalty-free research license to any compounds or products that we provide to St. Jude in connection with the research program, solely for academic research purposes. St. Jude is not permitted to license or sublicense such compounds or products or commercially exploit them in any manner. The SRA will continue for a period of two years unless earlier terminated (i) by St. Jude if we fail to meet our material obligations under the agreement and do not cure such failure, (ii) by us if the principal investigator for the research

program is unable to supervise the research program and is not satisfactorily replaced by St. Jude, or (iii) by us if St. Jude fails to meet its material obligations under the agreement and does not cure such failure.

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UCSD Sponsored Research Agreement

On December 12, 2013, we entered into an agreement with The Regents of the University of California, on behalf of its San Diego Campus ("UCSD"), pursuant to which UCSD will undertake research projects related to a study on oxytocin. As consideration for the research program, we are obligated to pay an aggregate of approximately $1.6 million in fees to UCSD on a specified timeline, of which $0 has been paid as of the date hereof. This agreement will continue until completion of the projects, unless earlier terminated by either party (i) due to a material uncured breach of such agreement by the other party or (ii) for any reason by giving written notice to the other party.

License Agreement Obligations

Our license agreements with Novartis and Novartis AG and Dr. Weg require us to make annual maintenance and milestone payments to the counterparties thereto.

Funding Requirements

We believe that our available cash and short-term investments as of the date of this filing, together with the net proceeds of this offering, will be sufficient to fund our anticipated level of operations for at least the next 12 months. Our future financing requirements will depend on many factors, some of which are beyond our control. Factors affecting our financing requirements include, but are not limited to:

- the rate of progress and cost of our clinical trials, preclinical studies and other discovery and research and development activities;
- the timing of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- our ability to manufacture sufficient quantities of our products to meet expected demand;
- the costs of preparing, filing, prosecuting, maintaining and enforcing any patent claims and other intellectual property rights, including litigation costs and the results of this litigation;
- our ability to enter into collaboration, licensing or distribution arrangements and the terms and timing of these arrangements;
- the potential need to expand our business, resulting in additional payroll and other overhead expenses;
- the potential need to acquire, by acquisition or in-licensing, other products or technologies; and
- the emergence of competing technologies or other adverse market or technological developments.

Future capital requirements will also depend on the extent to which we acquire or invest in additional complementary businesses, products and technologies. We currently have no understandings, commitments or agreements relating to any of these types of transactions.

At this time, we do not have sufficient capital to cover operating costs for the next twelve month period.

These conditions raise substantial doubt about the Company's ability to continue as a going concern. These condensed consolidated financial statements do not include any adjustments relating to the recovery of assets or the classification of liabilities that might be necessary should the Company be unable to continue as a going concern.

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Cash Flows

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

	For the nine months ended September 30,		For the year ended December 31, 2012	For the period from March 11, 2011 (inception) through December 31, 2011
	2013	2012		
Net cash used in operating activities	$(9,442,442)	$ (2,088,811)	$(2,736,739)	$(785,747)
Net cash used in investing activities	(6,670,416)	(1,569,018)	(1,699,593)	(12,872)
Net cash provided by financing activities	29,511,295	3,649,965	4,437,667	808,672
Net increase (decrease) in cash	13,398,437	(7,864)	1,335	10,053
Cash, beginning of period	11,388	10,053	10,053	—
Cash, end of period	$13,409,825	$ 2,189	$11,388	$10,053

Cash Flows from Operating Activities

Operating activities used approximately $2.74 million of cash during the year ended December 31, 2012 compared to $0.79 million from the period March 11, 2011 (inception) through December 31, 2011, the increase of approximately $1.95 million was primarily the result of the increase in net loss of approximately $27.07 million due to the significant expenses we incurred mainly for stock base compensation, compensation expense, and professional fees, offset by a non-cash charge increase of approximately $22.51 million as well as a net change of approximately $2.61 million in our accounts payable and accrued expenses. Non-cash charges consisted of stock base compensation granted to employees and consultants for services render in the amount of approximately $20.43 million. The net change in our operating assets and liabilities was primarily the result of accrued compensation expense.

Operating activities used approximately $9.4 million of cash during the nine month period ended September 30, 2013 compared $2.1 million for the nine month period ended September 30, 2012. The increase of $7.4 million was the result of a decrease in non-cash charges of $3.8 million, a net change in operating assets and liabilities of $283,187 and an increase in net loss of $3.9 million. Included in cash flows from operating activities is a registration payment obligation expense and reversal of the registration payment obligation liability of $360,000.

Cash Flows from Investing Activities

Cash used in investing activities for the year ended December 31, 2012 was approximately $1.70 million, compared to approximately $0.13 million from the period March 11, 2011 through December 31, 2011. The increase of approximately $1.57, million was primarily the result of $1.17 million to purchase intangible assets, primarily related sublicense from Ligand of the technology used in sparsentan.

Cash used in investing activities for the nine month period ended September 30, 2013 was $6.7 million compared to $1.6 million for the nine month period ended September 30, 2012. The increase of $5.1 million was primarily the result of repayment of a technology license liability of $1.3 million, a net purchase of marketable securities of $3.1 million, payments to secure exclusivity rights of certain licenses of $2.3 million, an increase in the purchase of fixed assets of $13,772, and an increase in our security deposit of $40,000, offset by a decrease in the purchase of intangible assets of $1.2 million, payments made on loans to stockholder of $399,329, and a decrease in a related party note receivable of $2,800.

Cash Flows from Financing Activities

For the year ended December 31, 2012, financing activities provided approximately $4.44 million, compared to proceeds of approximately $0.8 million from the period March 11, 2011 through December 31, 2011. The increase of approximately $3.6 million was primarily a result of an increase of approximately $2.7 million of proceeds from the private sale of our equity securities and approximately $0.9 million of proceeds from related parties' notes payable. For the nine month period ended September 30, 2013, cash provided by financing activities was $29,511,295 compared to $3,649,965 during the nine month period ended September 30, 2012. The increase of $25,861,330 was primarily a result of an increase of $28,590,254 in proceeds received from the private sale of our equity securities, offset by a registration payment of $946,196, and a decrease in activities associated with related party notes payable of $1,782,728.

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2013 Private Placements

In January 2013, we sold an aggregate of 272,221 shares of common stock at $3.00 per share in certain private placement transactions, for an aggregate purchase price of $816,664 in cash. The issuance of such shares of common stock was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

On February 14, 2013, we closed a private placement of 3,045,929 shares of our common stock, at a purchase price of $3.00 per share, or $9,137,787 in the aggregate, and Warrants to purchase up to an aggregate of 1,597,969 shares of common stock with an exercise price of $3.60 per such share underlying any warrant. We incurred fees of $678,986 in relation to the financing. The issuance of the shares of common stock in such private placement was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

On August 15, 2013, we closed a private placement and sold 5,531,401 shares of our common stock, at a purchase price of $4.50 per share, or $24,891,303 in the aggregate, and warrants to purchase up to an aggregate of 2,765,701 shares of common stock with an exercise price of $6.00 per share underlying each warrant. We incurred fees of $2,811,313 in relation to the financing. The issuance of the shares of common stock in such private placement was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

We entered into registration rights agreements concurrently with the closings of the February 2013 and August 2013 private placements, each of which required us to file a registration statement on Form S-1 within 30 days of the closing date of the transaction and cause such registration statement to be declared effective within 60 days thereafter. Each registration rights agreement provides for the payment of certain liquidated damages at the rate of 2% of the gross proceeds per month for each in which we are not in compliance with such agreement, not exceeding 10% of gross proceeds in the aggregate. As described elsewhere herein, we were not in compliance with the registration payment arrangement for the February 2013 registration rights agreement and therefore recorded $360,000 as registration payment obligation treated as a reduction of the proceeds received in the February financing transaction. We and the investors in the February 2013 private placement entered into the amended registration rights agreement, pursuant to which we paid an aggregate fee to such investors of $2.5 million. Additionally, we paid $103,425 to an investor to whom we sold shares in the January 2013 private placement and who participated in the August 2013 private placement. We recorded the aggregate amount of the payments made to the investors by to allocating approximately $360,000 to the waiver of the original registration payment obligation taken as a charge to operations and the remaining amount of $2,238,681 is treated as reduction of the proceeds received in the August 2013 private placement.

On September 13, 2013, we submitted a resale registration statement on Form S-1 to the SEC on a confidential basis. On December 6, 2013, the SEC declared the resale registration statement effective prior to the expiration of the contractually defined time period.

Subsequent Events

On October 1, 2013 we entered into a building lease for approximately 4,232 square feet of office space located in Cambridge, MA under which we are responsible for approximately $216,000 of annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes.

On December 1, 2013, we entered into a lease for approximately 2,500 square feet of office space located in San Diego, CA that expires in February, 2017. We are responsible for approximately $70,500 of annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes.

On December 6, 2013, our board of directors established a compensation policy for our non-employee directors pursuant to which each non-employee director shall receive $100,000 annually, which amount shall be comprised of not more than $25,000 in cash, with the remainder paid in the form of options to purchase shares of our common stock. Each non-employee director may, at his discretion, determine to receive less than $25,000 annually in the form of cash, in which case such amount will be paid to such director in the form of options to purchase additional shares of our common stock. In accordance with such policy, in December 2013, we issued options to purchase 51,000 shares of common stock to four non-employee directors. Such options vest immediately and are exercisable over a ten year period at an exercise price of $8.70 per share.

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On December 12, 2013, we entered into an agreement with UCSD pursuant to which UCSD will undertake research projects related to a study on oxytocin. As consideration for the research program, we are obligated to pay an aggregate of approximately $1.6 million in fees to UCSD on a specified timeline, of which $0 has been paid as of the date hereof. This agreement will continue until completion of the projects, unless earlier terminated by either party (i) due to a material uncured breach of such agreement by the other party or (ii) for any reason by giving written notice to the other party.

On December 12, 2013, we entered into an agreement with Novartis and Novartis AG pursuant to which Novartis and Novartis AG agreed to grant us an exclusive, perpetual, and royalty-bearing license for the manufacture, development and commercialization of Syntocinon and related intranasal products in the United States. Under the license, Novartis and Novartis AG are obligated to transfer to us certain information that is necessary for or related to the development or commercialization of Syntocinon. We are responsible for conducting research and preclinical, clinical and other development of Syntocinon at our own expense, and must use commercially reasonably efforts to develop Syntocinon in the United States.

As consideration for the license, we paid to Novartis and Novartis AG a $5 million upfront fee and are required to make substantial payments upon the achievement of certain milestones. Should we commercialize Syntocinon, we will be obligated to pay Novartis and Novartis AG a 20% royalty on net sales of such products. We are also required to pay annual maintenance fees to Novartis and Novartis AG. The license agreement contains other customary clauses and terms as are common in similar agreements in the industry.

On December 12, 2013, we entered into the Weg License Agreement pursuant to which Dr. Weg agreed to grant us an exclusive worldwide license for the manufacture, development and distribution of products to be developed for the treatment of central nervous system disorders. As consideration for the license, we are required to pay Dr. Weg an upfront fee, which amount included a $250,000 payment prior to the execution of the Weg License Agreement, as well as certain maintenance and sublicensing fees. We are also obligated to pay Dr. Weg certain royalties on sales of FDA-approved products.

The Weg License Agreement contains other customary clauses and terms as are common in similar agreements in the industry.

On December 16, 2013, we announced that we had withdrawn our proposal to acquire all of the issued and outstanding shares of common stock of Transcept Pharmaceuticals, Inc., or Transcept. The Company no longer owns any shares of Transcept’s common stock.

On December 16, 2013, we entered into the Shkreli Employment Agreement (as further described below) pursuant to which Mr. Shkreli will continue to serve as our Chief Executive Officer and we will pay Mr. Shkreli an annual base salary in the amount of $300,000 (subject to adjustments at the discretion of the Board after each anniversary of the Effective Date), and, at the sole discretion of our board of directors, an annual bonus award based upon specific goals and performance metrics.

In the fourth quarter of 2013 and early in the first quarter of 2014, we repurchased approximately 264,000 shares of our common stock for an aggregate purchase price of approximately $1.9 million. We currently recognize such repurchased shares of common stock as treasury stock.

On December 23, 2013, we entered into, and consummated the transactions contemplated by, a stock purchase agreement, which we refer to as the Kyalin Agreement, with Kyalin Biosciences, Inc., a Delaware corporation that we refer to as Kyalin, and the sellers signatory thereto, pursuant to which we acquired all of the issued and outstanding shares of capital stock of Kyalin. In consideration for such acquisition, we agreed to pay to the sellers (i) $1 million of cash consideration at specified dates; and (ii) up to $4 million of our common stock at certain dates and subject to the achievement of certain milestones. Under certain limited circumstances, we may be required to pay to the sellers, in the place of such shares of common stock, an amount of cash equal to one-half (1/2) of the value of the shares of common stock issuable in accordance with the Kyalin Agreement. In connection with such acquisition, we hired Srinivas Rao, M.D., Ph.D., the Founder and President of Kyalin.

Critical Accounting Policies

The preparation of financial statements in conformity with accounting principles generally accepted in the United States requires management to make estimates and assumptions that affect reported amounts of assets and liabilities as of the date of the balance sheet and reported amounts of expenses for the periods presented. Judgments must also be

made about the disclosure of contingent liabilities. Accordingly, actual results could differ significantly from those estimates. We believe the following discussion addresses the accounting policies that are necessary to understand and evaluate our reported financial results.

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Share-Based Payments

We adopted authoritative accounting guidance which establishes standards for share-based transactions in which we receive consultants or employee's services in exchange for equity instruments, such as stock incentive awards. These authoritative accounting standards require that we expense the fair value of stock awards, as measured on the awards' grant date.

If factors change and we employ different assumptions in the application of the relevant accounting guidance in future periods, the compensation expense that we record may differ significantly from what we have recorded in the current period. There is a high degree of subjectivity involved when using fair value to estimate share-based compensation. Consequently, there is a risk that our estimates of the fair values of our share-based compensation awards on the grant dates may bear little resemblance to the actual values realized upon the vesting, expiration, early termination or forfeiture of those share-based payments. Stock incentive awards options may expire worthless or otherwise result in zero value as compared to the fair values originally estimated on the grant date and reported in our financial statements. Alternatively, value may be realized from these instruments that are significantly in excess of the fair values originally estimated on the grant date and reported in our financial statements.

Income Taxes

We follow 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.

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, we 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. At the date of adoption, and as of September 30, 2013 and December 31, 2012, the Company does not have a liability for unrecognized tax uncertainties.

Our policy is to record interest and penalties on uncertain tax positions as income tax expense. As of and for the nine month period ended September 30, 2013 and the fiscal year end December 31, 2012, we had no accrued interest or penalties related to uncertain tax positions.

Impairment of long-lived assets

The Company periodically reviews long-lived assets for impairment whenever events or changes in circumstances indicate that the carrying amount of the assets may not be fully recoverable in accordance with ASC 360-10, "Impairment or Disposal of Long-Lived Assets". The Company recognizes an impairment loss when the sum of expected undiscounted future cash flows is less than the carrying amount of the asset. The amount of impairment is measured as the differences between the discounted future cash flow or estimated fair value and the book value of the underlying asset.

Derivative Instruments

The Company does not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. The Company evaluates all of its financial instruments to determine if such instruments are derivatives or contain features that qualify as embedded derivatives. For derivative financial instruments that are accounted for as liabilities, the derivative instrument is initially recorded at its fair value and is then revalued at each reporting date, with changes in the fair value reported in the statements of operations. For stock-based derivative financial instruments, the Company calculates the fair value of the financial instruments using a probability-weighted Black-Scholes option pricing model, which is comparable to the Binomial Lattice options pricing model at inception and on each

subsequent valuation date. The classification of derivative instruments, including whether such instruments should be recorded as liabilities or as equity, is evaluated at the end of each reporting period.

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Registration Payment Arrangement

The Company accounted for registration rights agreements in accordance with ASC 825-20, “Registration Payment Arrangements.” ASC 825-20 addresses an issuer’s accounting for registration payment arrangements. This pronouncement specifies that the contingent obligation to make future payments or otherwise transfer consideration under a registration payment arrangement, whether issued as a separate agreement or included as a provision of a financial instrument, should be separately recognized and accounted for as a contingency in accordance with ASC 450-20 “Loss Contingencies”.

Net loss per share

Basic net loss per common share is computed by dividing net loss applicable to common stockholders by the weighted average number of common shares outstanding during the periods presented as required by FASB ASC 260, (“Earnings Per Share”).

Patents

The Company capitalized external cost, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company expense cost associated with maintaining and defending patents subsequent to their issuance in the period incurred. The Company amortizes patent cost once issued on a straight-line basis over the estimate useful lives of the patents. The Company assess the potential impairment to all capitalized patent cost when events or changes in circumstances indicate that the carrying amount of our patent may not be recoverable. The Company accounts for patent costs in accordance with ASC Topic 350, “Goodwill and Other Intangible Assets” (“ASC 350”) and ASC Topic 805, “Business Combinations” (“ASC 805”).

Financial Instruments and Fair Value

ASC Topic 820, “Fair Value Measurements and Disclosures,” (“ASC Topic 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 Topic 820 are described below:

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

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

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

In estimating the fair value of the Company’s marketable securities available-for-sale, the Company used quoted prices in active markets.

In estimating the fair value of the Company’s derivative liabilities, the Company used a probability-weighted Black-Scholes option pricing model.

Financial assets with carrying values approximating fair value include cash as well as marketable securities, deposits on license agreements, prepaid expenses and other current assets. Financial liabilities with carrying values approximating fair value include accounts payable and accrued expenses.

Recently Issued Accounting Pronouncements

In February 2013, the FASB issued Accounting Standards Updated (“ASU”) 2013-04 “Obligations Resulting from Joint and Several Liability Arrangements for Which the Amount at the Reporting Date is Fixed”). The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount the entity has (a) contractually agreed to pay, and (b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company early adopted this guidance in the second quarter of 2013.

Except as noted above, we have evaluated recent accounting pronouncements and their adoption has not had or is not expected to have a material impact on our financial position or operations.

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Emerging Growth Company Critical Accounting Policy Disclosure

We qualify as an "emerging growth company" under the 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.

Controls and Procedures

Disclosure Controls and Procedures

We evaluated the effectiveness of our "disclosure controls and procedures" (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) as of September 30, 2013, which we refer to as the "Evaluation Date," and have concluded that as of the Evaluation Date, our disclosure controls were not effective to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act (i) is recorded, processed, summarized and reported, within the time periods specified in the SEC rules and forms and (ii) is accumulated and communicated to our management as appropriate to allow timely decisions regarding required disclosure.

Internal Controls

As of December 31, 2012, we identified certain matters that constitute a material weaknesses in our internal controls over financial reporting, specific material weaknesses include 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. We have taken certain steps in an effort to correct these material weaknesses, including, among other things hiring of a Chief Financial Officer who has significant experience with publicly held companies, hiring a controller to further segregate duties within the Company, appointing Cornelius ("Neal") E. Golding, who has more than 44 years of experience in finance and accounting, as an independent member of our board of directors and will also serve as the Chairman of the Audit Committee of our board of directors.

Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

In connection with the closing of the 2012 Merger, Marcum LLP Certified Public Accountants, the independent registered public accounting firm for former Retrophin, our predecessor, prior to the 2012 Merger, became the independent registered public accounting firm for us. On October 29, 2012, we filed a Current Report on Form 8-K with the SEC acknowledging the dismissal of Michael F. Cronin CPA as our independent registered public accounting firm due to the requirements of the SEC and the Public Company Accounting Oversight Board that lead and concurring reviewer partners cannot audit the same company for more than five consecutive years. Required disclosures in such Current Report on Form 8-K relating to our dismissal of the former accountant as required under Item 4.01, including the former accountants' letter of response to such dismissal, is incorporated herein by reference. The decision to appoint Marcum LLP was recommended, and subsequently approved, by our board of directors in connection with the 2012 Merger.

Quantitative and Qualitative Disclosures about Market Risk

Our primary exposure to market risk is related to changes in interest rates. As of September 30, 2013, we had cash and short-term investments of approximately $13.4 million, consisting of money market funds, U.S. treasuries and certificates of deposit. This exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term marketable securities. Our short-term investments are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10 percent change in interest rates would not have a material effect on the fair market value of our portfolio. We have the ability to hold our short-term investments until maturity, and therefore we would not expect our operations results or cash flows to be affected by any significant degree by the effect of a change in market interest rates on our investments. We carry our investments based on publicly available information. We do not currently have any hard to value investment securities or securities for which a market is not readily available or active.

We are not subject to significant credit risk as this risk does not have the potential to materially impact the value of our assets and liabilities.

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Corporate History

We were incorporated as Desert Gateway, Inc., an Oklahoma corporation, on February 8, 2008. Desert Gateway was originally a wholly owned subsidiary of American Merchant. In a 2008 reorganization of American Merchant, each share of outstanding common stock of American Merchant was converted into one share of Desert Gateway, while all of American Merchant's operating assets, liabilities and tax attributes (including accumulated losses and net operating losses) carried forward to another subsidiary of American Merchant in a downstream merger with such other subsidiary. Accordingly, American Merchant is not considered a predecessor company of the Company for accounting or legal purposes. Following the 2008 reorganization, Desert Gateway re-domiciled to Delaware. Since inception and until Desert Gateway's merger with Retrophin in December 2012 (as described below), Desert Gateway had no existing operations, and its sole purpose was to locate and consummate a merger or acquisition with a private entity. On December 12, 2012, Desert Gateway completed a merger, in which the former Retrophin became a wholly-owned subsidiary and the principal operating subsidiary of the Company.

On February 14, 2013, we changed our name to "Retrophin, Inc." through a short-form merger pursuant to Section 253 of the Delaware General Corporation Law, with its then wholly owned subsidiary, and our predecessor, former Retrophin, with us continuing as the surviving corporation following the merger. On April 1, 2013, our Board of Directors determined to change our fiscal year from a fiscal year ending in February of each year to a fiscal year ending on December 31 of each year.

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BUSINESS

Overview

We are a development-stage biopharmaceutical company focused on the development, acquisition and commercialization of therapies for the treatment of serious, catastrophic or rare diseases. We are developing Syntocinon TM Nasal Spray in the U.S. to assist initial postpartum milk ejection, and for the treatment of Schizophrenia and Autism. Syntocinon Nasal Spray is currently marketed by Novartis and Sigma-Tau in Europe and other countries for aiding milk let-down. In addition, we are developing RE-034, a synthetic hormone analogue that is composed of the first 24 amino acids of the 39 amino acids contained in ACTH for the treatment of IS and NS. We are developing RE-024, a novel small molecule, as a potential treatment for PKAN. Also, we are developing sparsentan, formerly known as RE-021, a dual acting receptor antagonist of angiotensin and endothelin receptors, for the treatment of FSGS. We also have several additional programs in preclinical development, including RE-001, a therapy for the treatment of DMD.

The following summarizes the status of our product candidates and preclinical programs, each of which will be described and discussed in further detail below under "—Our Product Candidates and Preclinical Programs."

- Syntocinon™ Nasal Spray. (oxytocin nasal spray, USP).

- Syntocinon Nasal Spray in Milk Let-Down. We intend to reintroduce Syntocinon to the U.S. market to assist initial postpartum milk ejection from the breasts. Disruption of oxytocin plays an important role in preventing the release of milk from the lactating breast. Numerous psychological and chemical stressors have been implicated in the inhibition of oxytocin release in new mothers resulting in impaired milk-ejection. There are currently no FDA-approved drugs for the treatment of milk let-down in the U.S. We believe that reintroduction of intranasal oxytocin would provide a convenient therapy for new mothers suffering from lactation deficiency.

- Syntocinon Nasal Spray in Schizophrenia. We intend to develop Syntocinon as a potential treatment for Schizophrenia. Current pharmaceutical treatment is limited to powerful antipsychotics with serious side effects and compliance problems. According to the National Institute of Mental Health, approximately one percent of Americans suffer from Schizophrenia. Over the past four years, three randomized, double-blind, placebo-controlled, independent proof-of-concept Schizophrenia trials were held. In all three trials, patients were highly symptomatic despite receiving therapeutic doses of an atypical antipsychotic. We believe that the findings of these studies suggest that intranasal oxytocin administered as an adjunct to subjects' antipsychotic drugs will improve positive and negative symptoms. We are partially funding a Phase 2 clinical study regarding the effects of oxytocin on the treatment of Schizophrenia. This trial is currently enrolling patients, and we expect approximately 143 patients to be enrolled. We expect results from this trial in the third quarter of 2014.

- Syntocinon Nasal Spray in Autism Spectrum Disorders. We also plan to develop Syntocinon for the potential treatment of symptoms in patients with Autism Spectrum Disorders. Approximately one in fifty children in the U.S. suffers from Autism Spectrum Disorders according to the Center for Disease Control and Prevention. Risperidone and aripiprazole are the only approved treatments for the behavioral disturbances associated with Autism. Common adverse effects from these drugs include weight gain, sedation, and extrapyramidal symptoms. Recent small clinical studies suggest that oxytocin may improve social cognition and quality of

life in patients with Autism. We believe that these studies support the development of Syntocinon for this indication. We intend to initiate a Phase 2 clinical study of Syntocinon for the treatment of Autism Spectrum Disorders in 2014.

- RE-034 (Tetracosactide Zinc).

- RE-034 in Infantile Spasms. IS, also known as West syndrome, is a form of epileptic encephalopathy that begins in infancy. IS is considered a catastrophic form of epilepsy due to the difficulty in controlling seizures and normalization of electroencephalography in addition to strong association with sequelae of developmental delay and mental retardation. Commercially available ACTH formulations that are substantially similar to RE-034 have been shown to be an effective treatment of IS. We intend to initiate a Phase 3 clinical trial of RE-034 for the treatment of IS in 2014.

- RE-034 in Nephrotic Syndrome. We intend to initiate studies of RE-034 for the treatment of NS. NS is a kidney disorder that leads to proteinuria, a condition in which an excess of proteins are contained in a patient's urine. Long-term conventional immunosuppression therapies have been used effectively to induce remission of proteinuria; however, many patients with NS will relapse after remission or are resistant to

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primary and secondary treatments. Commercially available ACTH formulations that are substantially similar to RE-034 have been shown to successfully induce remission of proteinuria in patients with NS. We intend to initiate a Phase 3 clinical trial of RE-034 for the treatment of NS in 2014.

- RE-024. We are developing RE-024, a novel small molecule, as a potential treatment for PKAN (Pantothenate Linase Associated Neurodegeneration). PKAN is the most common form of neurodegeneration with brain iron accumulation (NBIA). Classic PKAN is a genetic disorder that is typically diagnosed in the first decade of life. Consequences of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. PKAN is estimated to affect 1 to 3 persons per million. PKAN typically manifests in childhood with a profound, progressive dystonia and is usually lethal. There are currently no viable treatment options for patients with PKAN. RE-024 is a phosphopantothenate prodrug replacement therapy with the goal of restoring the supply of this operative substrate in PKAN patients. A Phase 1 clinical trial of RE-024 is expected to begin in early 2014 under an emergency IND.

- Sparsentan. Sparsentan is an investigational therapeutic agent which acts as both a potent angiotensin receptor blocker, or ARB, which is a type of drug that modulates the renin-angiotensin-aldosterone system and is typically used to treat hypertension, diabetic nephropathy and congestive heart failure, as well as a selective endothelin receptor antagonist, or ERA, which is a type of drug that blocks endothelin receptors, preferential for endothelin receptor type A. We have secured a license to sparsentan from Ligand and Bristol-Myers Squibb. We are developing sparsentan as a treatment for FSGS. FSGS is a leading cause of end-stage renal disease and NS. We are currently enrolling patients for a Phase 2 clinical study of sparsentan for the treatment of FSGS and we expect approximately 100 patients to be enrolled.

Our Strategy

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

- Expand our product pipeline by pursuing additional acquisitions of pharmaceutical products that have a profound impact on patients' lives. We believe that there are multiple drugs for treating life-threatening diseases that may be neglected by other pharmaceutical companies. We believe that we can acquire certain of these niche products to achieve increased sales.

- Focus on developing products to treat orphan or severe diseases. We focus on novel, life-saving orphan drug candidates in order to take advantage of our competitive strengths. We believe that drug development for orphan drug markets is particularly attractive because relatively small clinical trials can provide meaningful information regarding patient response and safety. Furthermore, the path to regulatory approval and commercial success for orphan drugs is less risky for an effective therapy, as compared to non-orphan drugs. Though we do not currently have any orphan drug designated product candidates, we expect to seek orphan drug designations from the FDA for RE-034, sparsentan, RE-024 and RE-001. However, there can be no assurance that the FDA will grant orphan status for such product candidates. Finally, we believe that our capabilities are well suited to the orphan drug market and represent distinct competitive advantages. Our management team and scientific staff, including Horacio Plotkin, our Chief Medical Officer, Andrew Vaino, our Vice President of Scientific Affairs, and Steve Eby, our Vice President of Global Strategy and Program Management, focus significantly on finding and developing treatments for orphan diseases and have

significant experience and expertise in drug technologies.

- Develop a sustainable pipeline by employing disciplined decision criteria. We seek to build a sustainable product pipeline by employing multiple therapeutic approaches and by developing or acquiring orphan drug candidates. We employ disciplined decision criteria to assess drug candidates, favoring drug candidates that have undergone at least some clinical study. Our decision to license a drug candidate will also depend on the scientific merits of the technology: the costs of the transaction and other economic terms of the proposed license; the amount of capital required to develop the technology; and the economic potential of the drug candidate, should it be commercialized. We believe this strategy minimizes our clinical development risk and allows us to accelerate the development and potential commercialization of current and future drug candidates. We intend to pursue regulatory approval for a majority of our drug candidates in multiple indications.

- Evaluate the commercialization strategies on a product-by-product basis to maximize the value of each. As we move our drug candidates through development toward regulatory approval, we will evaluate several options for each drug candidate's commercialization strategy. These options include building our own internal sales force; entering into joint marketing partnerships with other pharmaceutical or biotechnology companies, whereby we jointly sell and market the product; and out-licensing our products, whereby other pharmaceutical or

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biotechnology companies sell and market our product and pay us a royalty on sales. Our decision will be made separately for each product and will be based on a number of factors including capital necessary to execute on each option, size of the market and terms of potential offers from other pharmaceutical and biotechnology companies.

Our Product Candidates and Preclinical Programs

The following table summarizes the status of our product candidates and preclinical programs, each of which will be described and discussed in further detail below.

Syntocinon Nasal Spray

Syntocinon (oxytocin nasal spray, USP) is our product candidate for aiding milk let-down and for the treatment of Schizophrenia and Autism. Syntocinon is currently sold in Europe and other countries by Novartis and Sigma-Tau to aid mothers experiencing problems with milk let-down. Oxytocin is a nonapeptide hormone synthesized by the brain and released by the pituitary gland.

Oxytocin administration is known to have peripheral and central effects in humans. Commercially available intravenous oxytocin, sold under the brand name Pitocin and generically, is currently used in obstetrics for the induction of labor and postpartum hemorrhaging. Oral dosing of oxytocin is not a viable administration route given that polypeptides are inactivated in the gastroinstinal tract and liver. Nasal administration of oxytocin overcomes this therapeutic barrier. Intranasal oxytocin has been used to facilitate the milk let-down reflex. In addition, preclinical evidence suggests that oxytocin has a critical role in the regulation of brain-mediated processes that are involved in neuropsychiatric disorders. Clinical studies suggest that oxytocin may have positive effects on the treatment of symptoms in patients with Schizophrenia and Autism Spectrum Disorders.

Syntocinon Nasal Spray was an FDA-approved product for aiding milk let-down. Syntocinon Nasal Spray was voluntarily withdrawn from sale by Novartis Pharmaceutical Corporation, or Novartis, in 1997 for commercial reasons. On December 12, 2013, we secured a royalty-bearing license from Novartis to the U.S. rights for Syntocinon Nasal Spray, including the intellectual property to develop, manufacture, and sell the product in the United States.

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Syntocinon Nasal Spray in Milk Let-Down

We intend to reintroduce Syntocinon to the U.S. market to assist initial postpartum milk ejection from the breasts. Disruption of oxytocin plays an important role in preventing the release of milk from the lactating breast. Numerous psychological and chemical stressors have been implicated in the inhibition of oxytocin release in new mothers resulting in impaired milk-ejection. There are currently no FDA-approved drugs for the treatment of milk let-down in the U.S. We believe that reintroduction of intranasal oxytocin would provide a convenient therapy for new mothers suffering from lactation deficiency.

Syntocinon Nasal Spray in Schizophrenia

We intend to develop Syntocinon as a potential treatment for Schizophrenia. Current pharmaceutical treatment is limited to powerful antipsychotics with serious side effects and compliance problems. According to the National Institute of Mental Health, approximately one percent of Americans suffer from Schizophrenia. Over the past four years, three randomized, double-blind, placebo-controlled, independent proof-of-concept Schizophrenia trials were held. In all three trials, patients were highly symptomatic despite receiving therapeutic doses of an atypical antipsychotic. We believe that the findings of these studies suggest that intranasal oxytocin administered as an adjunct to subjects' antipsychotic drugs will improve positive and negative symptoms. We are partially funding a Phase 2 clinical study regarding the effects of oxytocin on the treatment of Schizophrenia. This trial is currently enrolling patients, and we expect approximately 143 patients to be enrolled. We expect results from this trial in the third quarter of 2014.

In 2010, the University of California, San Diego Medical Center, conducted a randomized, double-blind, crossover study of intranasal oxytocin in 19 schizophrenia patients with residual symptoms despite being on a stable dose of at least one antipsychotic. Patients received three weeks of daily intranasal oxytocin (titrated to 40 IU twice a day) and placebo adjunctive to their antipsychotics. In the 15 patients who completed all the study visits, it was demonstrated that oxytocin significantly reduced scores on the Positive and Negative Symptom Scale, or PANSS, ($p < .001$) and Clinical Global Impression-Improvement Scale ($p < .001$) compared with placebo at the three-week end point. No benefit was seen at the early time points. Oxytocin was well tolerated and produced no adverse effects based upon patient reports or laboratory analysis.

In 2011, The University of North Carolina at Chapel Hill, conducted a randomized, placebo-controlled study testing the effects of twice daily intranasal oxytocin treatment for 14 days on psychotic symptoms and social cognition in patients with schizophrenia. PANSS scores declined significantly and several social cognition measures improved significantly or nearly significantly in oxytocin (N=11) but not placebo (N=9) recipients. The study suggests that, in addition to reducing classic psychotic symptoms, oxytocin may diminish certain social cognition deficits that are not improved by current antipsychotic medications.

In 2012, Tehran University of Medical Sciences, conducted an eight-week, randomized, double-blind, placebo-controlled study of the efficacy and tolerability of oxytocin intranasal spray given as an adjuvant to risperidone in patients with schizophrenia. The study enrolled forty patients aged 18-50 years with a diagnosis of schizophrenia who were on a stable dose of risperidone for a minimum of 1 month, and who were chronically partially responsive to antipsychotic monotherapy. The trial demonstrated that intranasal oxytocin as an adjunct to risperidone tolerably and efficaciously improves positive symptoms of schizophrenia. In addition, effects on negative and total psychopathology scores were statistically significant, but were deemed likely to be clinically insignificant.

Syntocinon Nasal Spray in Autism Spectrum Disorders

We also plan to develop Syntocinon for the potential treatment of symptoms in patients with Autism Spectrum Disorders. Approximately one in fifty children in the U.S. suffers from Autism Spectrum Disorders according to the Center for Disease Control and Prevention. Risperidone and aripiprazole are the only approved treatments for the behavioral disturbances associated with Autism. Common adverse effects from these drugs include weight gain, sedation, and extrapyramidal symptoms. Recent small clinical studies suggest that oxytocin may improve social cognition and quality of life in patients with Autism. We believe that these studies support the development of Syntocinon for this indication. We intend to initiate a Phase 2 clinical study of Syntocinon for the treatment of Autism Spectrum Disorders in 2014.

RE-034 (Tetracosactide Zinc)

RE-034 is a synthetic hormone analog of the first 24 amino acids of the 39 amino acids contained in ACTH, formulated together with zinc. RE-034 exhibits the same physiological actions as endogenous ACTH by binding to all five melanocortin receptors (MCR), resulting in its anti-inflammatory and immunomodulatory effects. In 2014, we plan to submit an Investigational New Drug application, or IND, for RE-034 for the treatment of IS and NS to the FDA.

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RE-034 in Infantile Spasms

IS, also known as West syndrome, is a form of epileptic encephalopathy that begins in infancy. IS is considered a catastrophic form of epilepsy due to the difficulty in controlling seizures and normalization of electroencephalography in addition to strong association with sequelae of developmental delay and mental retardation. Commercially available ACTH formulations that are substantially similar to RE-034 have been shown to be an effective treatment of IS. We intend to initiate a Phase 1 clinical trial of RE-034 for the treatment of IS in 2014.

RE-034 in Nephrotic Syndrome

We intend to initiate studies of RE-034 for the treatment of NS. NS is a kidney disorder that leads to proteinuria, a condition in which an excess of proteins are contained in a patient's urine. Long-term conventional immunosuppression therapies have been used effectively to induce remission of proteinuria; however, many patients with NS will relapse after remission or are resistant to primary and secondary treatments. Commercially available ACTH formulations that are substantially similar to RE-034 have been shown to successfully induce remission of proteinuria in patients with NS. We intend to initiate a Phase 1 clinical trial of RE-034 for the treatment of NS in 2014.

RE-024 for the treatment of PKAN

We are developing RE-024, a novel small molecule, as a potential treatment for PKAN. PKAN is the most common form of neurodegeneration with brain iron accumulation. Classic PKAN is a genetic disorder that is typically diagnosed in the first decade of life. Consequences of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. PKAN is estimated to affect 1 to 3 persons per million. PKAN typically manifests in childhood with a profound, progressive dystonia and is usually lethal. There are currently no viable treatment options for patients with PKAN. RE-024 is a phosphopantothenate prodrug replacement therapy with the goal of restoring the supply of this operative substrate in PKAN patients.

PKAN is caused by a genetic downregulation of the enzyme pantothenate kinase (PANK), via a mutation in the pantothenate kinase-2 gene. PANK is responsible for the conversion of pantothenic acid to 4'phosphopantothenic acid, a precursor to Coenzyme A (CoA) in the brain. Because PANK is required for the production of CoA, animals or humans with downregulated PANK are unable to produce as much CoA as needed, which gives rise to the pathogenesis of PKAN. CoA is involved in a range of important biochemical functions, including the citric acid cycle, steroid biosynthesis, and histone and tubulin acetylation. Retrophin's approach seeks to improve neurological outcomes by directly replacing in the brain a molecule missing from PKAN sufferers.

The reaction catalyzed by PANK is depicted in Figure 1.

Missing enzyme in PKAN

Figure 1: Reaction catalyzed by PANK

RE-024 is a preclinical investigational program. Retrophin is in the process of synthesizing a focused library of pantothenate phosphate prodrugs. We began in vitro testing of these molecules in 2013. Phase 1 clinical studies are expected to begin in 2014, and, with strong Phase 1/2 data, an NDA filing could occur as early as 2016.

Sparsentan

We are developing sparsentan as a treatment for focal segmental glomerulosclerosis (FSGS) and other nephropathies. Sparsentan is an investigational therapeutic agent which acts as both a potent angiotensin receptor blocker (ARB), which is a type of drug that modulates the renin-angiotensin-aldosterone system and is typically used to treat hypertension, diabetic nephropathy and congestive heart failure, as well as a selective endothelin receptor antagonist (ERA), which is a type of drug that blocks endothelin receptors, preferential for endothelin receptor type A.

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Sparsentan is an endothelin receptor blocker that is specific to endothelin receptor type A (ETA) and it is not predicted to have the complications of drugs that block endothelin receptor type B (ETB). The stimulation of ETB mitigates relaxation of the wall of the arteries. When the endothelin binds to the ETB receptors, fluid loss occurs through an increase in the volume of urine produced, which is associated with sodium loss in the urine, which results in lower blood pressure. A blockade of ETB will therefore lead to fluid retention (edema) and a risk of increased blood pressure. Sparsentan is designed to block ETA rather than ETB, which results in less risk of edema as a side effect of the treatment.

Sparsentan in FSGS

We intend to develop sparsentan as a treatment for FSGS. FSGS is a leading cause of end-stage renal disease (ESRD) and NS. There are no FDA-approved treatments for FSGS and the off-label armamentarium is limited to ARBs, steroids, and immunosuppressant agents, which we believe are only effective for some patients. We estimate that there are at least 40,000 FSGS patients in the United States.

We believe that FSGS as an indication would be eligible to receive orphan drug status from both the FDA and the EMEA. FSGS is similar to over a dozen other rare, but severe, nephropathies and glomerulopathies for which Sparsentan could serve a critical role. Retrophin believes that a drop in proteinuria could serve as a primary endpoint in a pivotal clinical study and that FDA approval could be received on the basis of a single, small pivotal trial.

RE-001 for the treatment of Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a severe form of muscular dystrophy characterized by rapid progression of muscle degeneration, eventually leading to loss of ambulation and death. DMD affects one in 3,500 males and is the most prevalent of the muscular dystrophies. DMD is caused by a mutation in the dystrophin gene, causing a downregulation of the dystrophin protein required for muscle cell structure. There is no known cure for DMD.

RE-001 is designed to be a recombinant, modified form of micro-utrophin, a protein similar to the dystrophin protein that is missing in the muscles of DMD patients. RE-001 is designed as micro-utrophin fused to a cell-penetrating peptide known as TAT, which is believed to allow for delivery of the modified form of utrophin into muscle cells, where it is needed for structural support. Pre-clinical studies of RE-001 in “mdx” mice (an animal model for DMD), resulted in reduced creatine kinase excretion, a marker of muscle damage, as well as increased muscle function and lifespan. Retrophin plans to develop RE-001 to treat DMD in humans.

Licenses and Royalties

Novartis License

On December 12, 2013, we entered into an agreement with Novartis and Novartis AG pursuant to which Novartis and Novartis AG agreed to grant us an exclusive, perpetual, and royalty-bearing license for the manufacture, development and commercialization of Syntocinon and related intranasal products in the United States. Under the license, Novartis and Novartis AG are obligated to transfer to us certain information that is necessary for or related to the development or commercialization of Syntocinon. We are responsible for conducting research and preclinical, clinical and other development of Syntocinon at our own expense, and must use commercially reasonably efforts to develop Syntocinon in the United States.

As consideration for the license, we paid to Novartis and Novartis AG a $5 million upfront fee and are required to make substantial payments upon the achievement of certain milestones. Should we commercialize Syntocinon, we will be obligated to pay Novartis and Novartis AG a 20% royalty on net sales of such products. We are also required to pay annual maintenance fees to Novartis and Novartis AG.

The license agreement contains other customary clauses and terms as are common in similar agreements in the industry. The license agreement will continue in perpetuity unless terminated by us or by Novartis and Novartis AG.

Ligand License

In February 2012, we entered into an agreement pursuant to which Ligand agreed to grant us a worldwide license for the development, manufacture and commercialization of sparsentan, an ARB and ERA which we are initially using in connection with the treatment of FSGS. Under the license agreement, Ligand granted us a sublicense under certain of its patents and other intellectual property in connection with the development and commercialization of sparsentan. Under the license agreement, Ligand is obligated to transfer to us certain information, records, regulatory filings, materials and

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inventory controlled by Ligand and relating to or useful for developing sparsentan. We must use commercially reasonable efforts to develop and commercialize sparsentan in specified major market countries and other countries in which we believe it is commercially reasonable to develop and commercialize such products.

As consideration for the license, we are required to make substantial payments payable upon the achievement of certain milestones totaling up to $106.9 million. Should we commercialize sparsentan or any products containing any of these compounds, we will be obligated to pay to Ligand an escalating annual royalty between 10% and 20% of net sales of all such products. Through September 30, 2013, we made payments to Ligand of $2.55 million under the license agreement.

In the event that we desire to enter into a license arrangement with respect to any licensed compound under the license agreement, Bristol-Myers Squibb Company will have a right of first negotiation and Ligand will have a right of second negotiation with respect to any such license arrangement for a licensed compound.

The license agreement contains other customary clauses and terms as are common in similar agreements in the industry.

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

Central Nervous System License

On December 12, 2013, the we entered into the Weg License Agreement with Stuart Weg, MD, pursuant to which Dr. Weg agreed to grant us an exclusive worldwide license for the manufacture, development and distribution of products to be developed for the treatment of central nervous system disorders. As consideration for the license, we are required to pay Dr. Weg an upfront fee, which amount included a $250,000 payment prior to the execution of the Weg License Agreement, as well as certain maintenance and sublicensing fees. We are also obligated to pay Dr. Weg certain royalties on sales of FDA-approved products.

The Weg License Agreement contains other customary clauses and terms as are common in similar agreements in the industry.

The Weg License Agreement will continue in perpetuity unless terminated by us or by Dr. Weg. The Company may terminate the agreement at any time by giving written notice to Dr. Weg. Dr. Weg may terminate the agreement due to the Company’s uncured material breach of the agreement.

Research Agreements

St. Jude Sponsored Research Agreement

Effective October 1, 2013, we entered into a sponsored research agreement with St. Jude, pursuant to which St. Jude will undertake a research program with respect to RE-024. As consideration for the research program, we are obligated to pay an aggregate of $780,674 in fees to St. Jude on a specified timeline, of which $195,168 has been paid as of the date hereof. Pursuant to this agreement, we granted St. Jude a non-exclusive, royalty-free research license to any compounds or products that we provide to St. Jude in connection with the research program, solely for academic research purposes. St. Jude is not permitted to license or sublicense such compounds or products or commercially exploit them in any manner. This agreement will continue for a period of two years unless earlier terminated (i) by St. Jude if we fail to meet our material obligations under the agreement and do not cure such failure, (ii) by us if the principal investigator for the research program is unable to supervise the research program and is not satisfactorily replaced by St. Jude, or (iii) by us if St. Jude fails to meet its material obligations under the agreement and does not cure such failure.

UCSD Sponsored Research Agreement

On December 12, 2013, we entered into an agreement with The Regents of the University of California, on behalf of its San Diego Campus (“UCSD”), pursuant to which UCSD will undertake research projects related to a study on oxytocin. As consideration for the research program, we are obligated to pay an aggregate of approximately $1.6 million in fees to UCSD on a specified timeline, of which $0 has been paid as of the date hereof. This agreement will continue until completion of the projects, unless earlier terminated by either party (i) due to a material uncured breach of such agreement by the other party or (ii) for any reason by giving written notice to the other party.

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Intellectual Property

We have secured a royalty-bearing license from Novartis to the U.S. rights for Syntocinon Nasal Spray, including the intellectual property to develop, manufacture, and sell the product in the United States. We have also secured a license to sparsentan, an ARB and ERA which we are initially using in connection with the treatment of FSGS from Ligand and Bristol-Myers Squibb.

We have licenses to an issued U.S. patent covering the sparsentan compound, a crystalline form of the compound, and pharmaceutical compositions of matter that include the sparsentan compound, which will currently expire in 2019 before any patent term extension. There are issued patents or pending applications covering sparsentan in at least the following foreign jurisdictions: Australia, Belgium, China, Denmark, Finland, France, Germany, Ireland, Japan, Luxembourg, Netherlands, Sweden, Switzerland and the United Kingdom. Outside the United States, the sparsentan patents and patent applications relate to compositions and methods of use. We have filed one application covering RE-024 in the United States (for which a notice of allowance was received in January 2014), and a PCT counterpart filing has been made. The allowed United States claims are directed to the RE-024 compound, pharmaceutical compositions of matter that include the RE-024 compound, methods of treatment using the RE-024 compound, and methods of preparing the RE-024 compound. We do not own or license any issued or pending applications for patents covering Syntocinon, RE-034 or RE-001. In jurisdictions which permit such, we will seek patent term extensions, for example as provided for in the Hatch-Waxman Act in the United States, where possible for certain of our patents. We plan to pursue additional patents in and outside of the United States covering additional therapeutic uses of sparsentan from these existing applications. In addition, we will pursue patent protection for any new discoveries or inventions made in the course of our development of sparsentan.

If we obtain marketing approval for sparsentan or other drug candidates in the United States or in certain jurisdictions outside of the United States, we may be eligible for regulatory protection, such as five years of new chemical entity exclusivity, seven years of orphan drug exclusivity and as mentioned below, up to five years of patent term extension potentially available in the United States under the Hatch-Waxman Act, eight to 11 years of data and marketing exclusivity potentially available for new drugs in the European Union, up to five years of patent extension in Europe (Supplemental Protection Certificate), and eight years of data exclusivity potentially available in Japan. There can be no assurance that we will qualify for any such regulatory exclusivity, or that any such exclusivity will prevent competitors from seeking approval solely on the basis of their own studies. See “Government Regulation” below.

Our goal is to obtain, maintain and enforce patent protection for our products, formulations, processes, methods and other proprietary technologies, preserve our trade secrets, and operate without infringing on the proprietary rights of other parties, both in the United States and in other countries. Our policy is to actively seek to obtain, where appropriate, the broadest intellectual property protection possible for our current product candidates and any future product candidates, proprietary information and proprietary technology through a combination of contractual arrangements and patents, both in the United States and abroad. However, even patent protection may not always afford us with complete protection against competitors who may seek to circumvent our patents. Our proprietary rights may not adequately protect our intellectual property and potential products, and if we cannot obtain adequate protection of our intellectual property and potential products, we may not be able to successfully market our potential products.

We will depend upon the skills, knowledge and experience of our scientific and technical personnel, as well as that of our advisors, consultants and other contractors, none of which is patentable. To help protect our proprietary know-how, which is not patentable, and inventions for which patents may be difficult to obtain or enforce, we will in the future rely on trade secret protection and confidentiality agreements to protect our interests. To this end, we plan to require all of our employees, consultants, advisors and other contractors to enter into confidentiality agreements that prohibit the disclosure of confidential information and, where applicable, require disclosure and assignment to us of the ideas, developments, discoveries and inventions important to our business.

Manufacturing

We intend to continue to use our financial resources to accelerate development of our drug candidates rather than diverting resources to establish our own manufacturing facilities. We intend to meet our pre-clinical and clinical trial manufacturing requirements by establishing relationships with third-party manufacturers and other service providers to perform these services for us. We do not have any long-term agreements or commitments for these services.

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

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Sales and Marketing

We currently have no commercial infrastructure. In order to commercialize our clinical drug candidates if and when they are approved for sale in the United States or elsewhere, we will need to build marketing, sales and distribution capabilities.

We may be subject to various federal and state laws pertaining to health care "fraud and abuse," including anti-kickback laws and false claims laws. Anti-kickback laws make it illegal for a prescription drug manufacturer to solicit, offer, receive, or pay any remuneration in exchange for, or to induce, the referral of business, including the purchase or prescription of a particular drug. Due to the breadth of the statutory provisions and the absence of guidance in the form of regulations and very few court decisions addressing industry practices, it is possible that our practices might be challenged under anti-kickback or similar laws. False claims laws prohibit anyone from knowingly and willingly presenting, or causing to be presented for payment to third-party payors (including Medicare and Medicaid) claims for reimbursed drugs or services that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services. Our activities relating to the sale and marketing of our products may be subject to scrutiny under these laws.

Pricing and Reimbursement

We expect a portion of our future end-user demand for our drugs, if approved, will come from patients covered under Medicaid, Medicare and other government-related programs such as TRICARE and the Department of Veterans Affairs, or the VA. As required by Federal regulations, we will need to provide rebates and discounts in connection with these programs. As a result of Medicaid rebates, we may not generate any net revenues with respect to Medicaid sales, but we may generate net revenues with respect to Medicare sales, TRICARE sales and sales made to the VA. In addition, it is possible that future legislation in the United States and other jurisdictions could be enacted which could potentially impact the reimbursement rates for the products we are developing and may develop in the future and also could further impact the levels of discounts and rebates paid to federal and state government entities. Any legislation that impacts these areas could impact, in a significant way, our ability to generate revenues from sales of products that, if successfully developed, we bring to market.

Competition

The pharmaceutical and biotechnology industries are intensely competitive and subject to rapid and significant technological change. Most of our competitors are larger than us and have substantially greater financial, marketing and technical resources than we have. If our business strategy is successful, we likely will attract additional competition.

The development and commercialization of new products to treat orphan diseases is highly competitive, and we expect considerable competition from major pharmaceutical, biotechnology and specialty pharmaceutical companies. As a result, there are, and will likely continue to be, extensive research and substantial financial resources invested in the discovery and development of new orphan drug products. Our potential competitors include, but are not limited to, Genentech, GlaxoSmithKline, Roche, Novartis, Pfizer, Boehringer Ingelheim, Sanofi, BioMarin, Sarepta, Vertex, and Jazz Pharmaceuticals.

There are also many companies, both public and private, including well-known pharmaceutical companies, which are engaged in the development of products for certain of the applications being pursued by Retrophin, such as Schizophrenia, Autism Spectrum Disorders, IS, NS, PKAN, FSGS and DMD.

For example, in June, 2013, Questcor Pharmaceuticals, Inc. entered into an agreement with Novartis to license Synacthen and Synacthen Depot, which may be used for IS and NS.

Clinical studies of Deferiprone as a potential treatment for PKAN, sponsored by ApoPharma Inc. and TIRCON ("Treat Iron-Related Childhood-Onset Neurodegeneration"), have been reported. Additionally, we believe that TIRCON is working on a possible treatment for PKAN using pantethine derivatives.

There are also clinical studies underway evaluating possible treatments for FSGS. For example, Sanofi (Genzyme) is engaged in a Phase 2 clinical study of Fresolimumab to treat FSGS, and Sunnybrook Medical Center has announced plans for a Phase 2 clinical study of Rituxan to treat FSGS. Also, Fibrogen is developing an anti-Connective Tissue Growth Factor (CTGF) antibody as a possible treatment for FSGS. The following biotechnology and pharmaceutical companies are working on developing potential treatments for DMD and have products which are currently in or have completed the following clinical stages: GlaxoSmithKline/Prosensa and Santhera/Takeda (Phase 3); Acceleron

Pharma/Shire, Sarepta Therapeutics, Phrixus, Prosensa and PTC Therapeutics (Phase 2); and Sarepta Therapeutics and Tivorsan Pharmaceuticals and possibly others (Preclinical). Additionally, several FDA approved drugs for other indications are being tested in clinical trials for DMD, including prednisone, sildenafil citrate (sold under the trademark Viagra, among others) and IGF-1.

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We are an early stage company with no history of operations. Many of our competitors have substantially more resources than we do, including both financial and technical. In addition, many of our competitors have more experience than us in pre-clinical and clinical development, manufacturing, regulatory and global commercialization. We are also competing with academic institutions, governmental agencies and private organizations that are conducting research in the field of orphan diseases.

Our competition will be determined in part by the potential indications for which drugs are developed and ultimately approved by regulatory authorities. Additionally, the timing of market introduction of some of our potential products or our competitors' products may be an important competitive factor. Accordingly, the speed with which we can develop products, complete pre-clinical testing, clinical trials, approval processes, and supply commercial quantities to market are expected to be important competitive factors. We expect that competition among products approved for sale will be based on various factors, including product efficacy, safety, reliability, availability, price, reimbursement, and patent position.

Clinical Testing of Our Products in Development

Each of our products in development, and likely all future drug candidates we develop, will require extensive pre-clinical and clinical testing to determine the safety and efficacy of the product applications prior to seeking and obtaining regulatory approval. This process is expensive and time consuming. In completing these trials, we are dependent upon third-party consultants, consisting mainly of investigators and collaborators, who will conduct such trials.

We and our third-party consultants conduct pre-clinical testing in accordance with Good-Laboratory Practices, or GLP, and clinical testing in accordance with Good Clinical Practice standards, or GCP, which are international ethical and scientific quality standards utilized for pre- clinical and clinical testing, respectively. GCP is the standard for the design, conduct, performance, monitoring, auditing, recording, analysis and reporting of clinical trials, and is required by the FDA to be followed in conducting clinical trials.

Government Regulation

FDA Approval Process

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

Pharmaceutical product development for a new product or certain changes to an approved product in the U.S. typically involves preclinical laboratory and animal tests, the submission to the FDA of an investigational new drug application, or IND, which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity, and novelty of the product or disease.

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

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted: (i) in compliance with federal regulations; (ii) in compliance with good clinical practice, or GCP, an international standard meant to protect the rights and health

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and to define the roles of clinical trial sponsors, administrators, and monitors; as well as (iii) under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

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

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

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the U.S. The NDA must include the results of all preclinical, clinical, and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture, and controls. The cost of preparing and submitting an NDA is substantial. The submission of most NDAs is additionally subject to a substantial application user fee, currently exceeding $2,169,000, and the manufacturer and/or sponsor under an approved new drug application are also subject to annual product and establishment user fees, currently exceeding $104,000 per product and $554,000 per establishment. These fees are typically increased annually.

The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of new drug applications. Most such applications for standard review drug products are reviewed within ten to twelve months; most applications for priority review drugs are reviewed in six to eight months. Priority review can be applied to drugs that the FDA determines offer major advances in treatment, or provide a treatment where no adequate therapy exists. The review process for both standard and priority review may be extended by FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission.

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

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

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An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

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

The Hatch-Waxman Amendments

Orange Book Listing

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

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

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

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

Exclusivity

Upon NDA approval of a new chemical entity or NCE, which is a drug that contains no active moiety that has been approved by FDA in any other NDA, that drug receives five years of marketing exclusivity during which FDA cannot receive any ANDA seeking approval of a generic version of that drug or any Section 505(b)(2) NDA, discussed in more detail below, that relies on the FDA's findings regarding that drug. A drug may obtain a three-year period of

exclusivity for a change to the drug, such as the addition of a new indication to the labeling or a new formulation, during which FDA cannot approve an ANDA or any Section 505(b)(2) NDA, if the supplement includes reports of new clinical studies (other than bioavailability studies) essential to the approval of the supplement.

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An ANDA may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. If there is no listed patent in the Orange Book, there may not be a Paragraph IV certification, and, thus, no ANDA may be filed before the expiration of the exclusivity period.

Patent Term Extension

After NDA approval, owners of relevant drug patents may apply for up to a five year patent extension. The allowable patent term extension is calculated as half of the drug's testing phase—the time between IND application and NDA submission— and all of the review phase—the time between NDA submission and approval up to a maximum of five years. The time can be shortened if FDA determines that the applicant did not pursue approval with due diligence. The total patent term after the extension may not exceed 14 years.

For patents that might expire during the application phase, the patent owner may request an interim patent extension. An interim patent extension increases the patent term by one year and may be renewed up to four times. For each interim patent extension granted, the post-approval patent extension is reduced by one year. The director of the United States Patent and Trademark Office must determine that approval of the drug covered by the patent for which a patent extension is being sought is likely. Interim patent extensions are not available for a drug for which an NDA has not been submitted.

Section 505(b)(2) NDAs

Most drug products obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on FDA's findings of safety and effectiveness in the approval of a similar product or published literature in support of its application.

Section 505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference. If the Section 505(b)(2) applicant can establish that reliance on FDA's previous findings of safety and effectiveness is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all, or some, of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

To the extent that the Section 505(b)(2) applicant is relying on the FDA's findings of safety and effectiveness for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. Thus approval of a Section 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant. As with traditional NDAs, a Section 505(b)(2) NDA may be eligible for three-year marketing exclusivity, assuming the NDA includes reports of new clinical studies (other than bioavailability studies) essential to the approval of the NDA.

Post-Approval Requirements

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

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, and surveillance to monitor the effects of an approved product, or the FDA may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality-control, drug manufacture, packaging, and labeling procedures must continue to conform to cGMPs, after approval. Drug manufacturers and certain of their subcontractors are required to register their

establishments with FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must

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continue to expend time, money, and effort in the areas of production and quality-control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Pediatric Information

Under the Pediatric Research Equity Act, or PREA, NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant full or partial waivers, or deferrals, for submission of data. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted.

The Best Pharmaceuticals for Children Act, or BPCA, provides NDA holders a six-month extension of any exclusivity—patent or non-patent—for a drug if certain conditions are met. Conditions for exclusivity include the FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, FDA making a written request for pediatric studies, and the applicant agreeing to perform, and reporting on, the requested studies within the statutory timeframe. Applications under the BPCA are treated as priority applications, with all of the benefits that designation confers.

Orphan Drugs

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

Fast Track Designation and Accelerated Approval

FDA is required to facilitate the development, and expedite the review, of drugs that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the fast track program, the sponsor of a new drug candidate may request that FDA designate the drug candidate for a specific indication as a fast track drug concurrent with, or after, the filing of the IND for the drug candidate. FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request.

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

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

In addition to other benefits such as the ability to use surrogate endpoints and engage in more frequent interactions with FDA, FDA may initiate review of sections of a fast track drug's NDA before the application is complete. This rolling review is

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available if the applicant provides, and FDA approves, a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, the fast track designation may be withdrawn by FDA if FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

Anti-Kickback, False Claims Laws

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce; or in return for; purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid, or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties, and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. This includes claims made to programs where the federal government reimburses, such as Medicaid, as well as programs where the federal government is a direct purchaser, such as when it purchases off the Federal Supply Schedule. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Other Federal and State Regulatory Requirements

The Centers for Medicare & Medicaid Services, or CMS, has issued a final rule that requires manufacturers of prescription drugs to begin collecting and reporting information on payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members. Manufacturers were required to begin collecting information on August 1, 2013, with the first reports due March 31, 2014. The reported data will be posted in searchable form on a public website beginning September 30, 2014. Failure to submit required information may result in civil monetary penalties.

In addition, several states now require prescription drug companies to report expenses relating to the marketing and promotion of drug products and to report gifts and payments to individual physicians in these states. Other states prohibit various other marketing-related activities. Still other states require the posting of information relating to clinical studies and their outcomes. In addition, California, Connecticut, Nevada, and Massachusetts require pharmaceutical companies to implement compliance programs and/or marketing codes. Several additional states are considering similar proposals. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties.

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Foreign Regulation

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

Other Laws and Regulatory Processes

We are subject to a variety of financial disclosure and securities trading regulations as a public company in the United States, including laws relating to the oversight activities of the Securities and Exchange Commission, or SEC, and, if our capital stock becomes listed on a national securities exchange, we will be subject to the regulations of such exchange on which our shares are traded. In addition, the Financial Accounting Standards Board, or FASB, the SEC, and other bodies that have jurisdiction over the form and content of our accounts, our financial statements and other public disclosure are constantly discussing and interpreting proposals and existing pronouncements designed to ensure that companies best display relevant and transparent information relating to their respective businesses.

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

Employees

As of the date of this report, we employed 22 employees, each of whom is full-time and five consultants provide significant assistance to us. To successfully develop our drug candidates, we must be able to attract and retain highly skilled personnel. We anticipate hiring up to 15 additional full-time employees devoted to development activities and up to 5 additional full-time employees for general and administrative activities over the next few years. In addition, we intend to use clinical research organizations and third parties to perform our clinical studies and manufacturing.

Organization and Consolidated Subsidiaries.

We do not have any active subsidiaries and all of our assets and operations are maintained by Retrophin.

Property

We lease our principal executive offices, which are located at 777 Third Avenue, 22 nd Floor, New York, NY 10017. We also lease 4,232 square feet of office space located in Cambridge, MA and approximately 2,500 square feet of office space located in San Diego, CA.

Legal Proceedings

We have no material proceedings pending nor are we aware of any pending investigation or threatened litigation by any third party.

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MANAGEMENT

The following table sets forth the name, age, and position of our directors and officers as of the date of this prospectus. Executive officers are elected annually by our board of directors. Each executive officer holds his office until he resigns, is removed by the board, or his successor is elected and qualified. Directors are elected annually by our stockholders at the annual meeting. Each director holds his office until his successor is elected and qualified or his earlier resignation or removal.

Name	Age	Position
Martin Shkreli	30	Chief Executive Officer and Director
Marc Panoff	43	Chief Financial Officer
Horacio Plotkin, M.D.	48	Chief Medical Officer
Stephen Aselage	62	Director
Steve Richardson	59	Director
Cornelius E. Golding	66	Director
Jeffrey Paley, M.D.	46	Director

MARTIN SHKRELI has served as the Chief Executive Officer and as a director of the Company since December 17, 2012. Previously, Mr. Shkreli was the founder of Retrophin, LLC (the predecessor of our predecessor, Retrophin, Inc.) and served as the President of our predecessor since its formation. Mr. Shkreli is also the founder and managing partner of MSMB Capital Management, a New York hedge fund firm founded in 2006 that manages a variety of partnerships. Prior to MSMB, Mr. Shkreli was employed at Intrepid Capital Management from 2004 to 2006 and previously at Cramer Berkowitz & Co, both of which are hedge fund firms based in New York. Mr. Shkreli is an experienced biotechnology and pharmaceutical industry investor, particularly in businesses with orphan drugs. Mr. Shkreli received his BBA from Baruch College. Mr. Shkreli was selected as a director because of his business and professional experience, including but not limited to his leadership of Retrophin in the early stages, private and public financings and a successful track record of identifying drug assets.

MARC PANOFF has served as the Chief Financial Officer of the Company since May 20, 2013. Prior to joining the Company and beginning in February 2012, Mr. Panoff served as a Senior Partner and Vice President of Finance at GroupM North America, the world’s number one media investment management group. From January 2006 to February 2012, Mr. Panoff served as Chief Financial Officer, Treasurer and Secretary of Neurologix, Inc., a publicly traded company that was engaged in the research and development of proprietary treatments for the brain and central nervous system, primarily utilizing gene therapies. On March 16, 2012, Neurologix filed a voluntary petition under Chapter 7 of the United States Bankruptcy Code in the state of Delaware. From July 2004 to January 2006, Mr. Panoff served as Chief Financial Officer of Nephros, Inc., a publicly traded medical device developer. Mr. Panoff received his Bachelor of Science in Business Administration from Washington University in St. Louis and his Masters in Business Administration from Arizona State University. He is also a Certified Public Accountant in New York State.

HORACIO PLOTKIN, M.D. has served as the Chief Medical Officer of the Company since May 13, 2013. Prior to joining the Company and beginning in 2012, Dr. Plotkin served as the Executive Medical Director of Clinical Research at Alexion Pharmaceuticals, Inc., a biotechnology company focused on delivering life-transforming therapies for patients suffering from ultra-rare, severe, and life-threatening disorders. From 2010-2011, Dr. Plotkin served as Senior Medical Director of Clinical Research at Enobia Phanna, Corp., a private biopharmaceutical company focused on the development of therapies to treat patients with ultra-rare and life-threatening genetic metabolic disorders, which was acquired by Alexion Pharmaceuticals on December 28, 2011. From 2008 to 2011, Dr. Plotkin served as Medical Director of Clinical Research at Genzyme Corporation, a biotechnology company, where Dr. Plotkin led his team to the approval of a treatment for Pompe disease. Dr. Plotkin will continue to serve as an Adjunct Associate Professor of Pediatrics and Orthopedic Surgery at the University of Nebraska School of Medicine, a position he has held since 2007. Dr. Plotkin earned his M.D. from the University of Buenos Aires School of Medicine in 1987.

STEPHEN ASELAGE has served as a director of the Company since December 17, 2012. Previously, Mr. Aselage was a director of our predecessor, Retrophin, Inc., since October 2012. Prior to joining Retrophin, Mr. Aselage served

as the Executive Vice President and Chief Business Officer at BioMarin, a biotechnology company, from December 2009 through September 2012. And from June 2005 to December 2009, Mr. Aselage served as BioMarin's Senior Vice President of Global Commercial Development. From February 2004 to June 2005, Mr. Aselage served as Executive Vice President of

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Global Commercial Operations at Cell Therapeutics, a biotechnology company focused on cancer therapeutics. From September 2003 to January 2004, Mr. Aselage served as Senior Vice President of North American Sales and Marketing for Genzyme Corporation, a biotechnology company, following Genzyme's acquisition of Sangstat Medical Corporation where he had worked since February 1999. While at Sangstat, Mr. Aselage restructured the company's sales, marketing and medical affairs groups. From 1996 through 1999, Mr. Aselage served as Director of Sales and Marketing at Advanced Tissue Sciences, a biotechnology company. Earlier in his career, Mr. Aselage held a variety of sales and sales management positions at biotechnology and pharmaceutical companies including Rhone-Poulenc Rorer Pharmaceuticals (now Sanofi-Aventis), Genentech, Inc., and Bristol Laboratories, a biopharmaceutical company. Mr. Aselage holds a B.S. in biology from the University of Notre Dame. Mr. Aselage was selected as a director because of his business and professional experience, including but not limited to his leadership of BioMarin in drug commercialization, private and public financings and a successful turnaround of multiple businesses.

STEVE RICHARDSON has been a director of the Company since December 17, 2012. Previously, Mr. Richardson was a Manager of Retrophin, LLC (the predecessor of Retrophin, Inc.) since June 2011. Mr. Richardson is a Senior Advisor to The Boston Consulting Group, a global management consulting firm, a position he has held since early 2009. Previously Mr. Richardson spent over 30 years with American Express, most recently as Senior Vice President of Human Resources and Chief Talent Officer, where he served as a key advisor for major business transformation and enterprise-wide organizational change and restructuring. Mr. Richardson served as a Board member of United Way Worldwide from 2008 to 2010 and is currently a Senior Advisor to the Center for Talent Innovation, a task force focused on identifying, developing and promoting a second generation of corporate policies and practices that support the ambition, work and life needs of highly qualified talent across the divides of gender, generation and culture. Mr. Richardson was selected as a director due to his extensive experience in overseeing and advising growing companies and substantial experience in business transformation, global general management and recruiting and developing talented management.

CORNELIUS E. GOLDING has served as a director of the Company since October 1, 2013. Previously, Mr. Golding was the Senior Vice President and Chief Financial Officer of Atlantic Mutual Insurance Company, where, among other responsibilities, he oversaw the corporate investment portfolio, a position he held from August 1994 to his retirement in September 2003. Previously, from 1981 to 1994, Mr. Golding served in various management and executive positions at Atlantic Mutual Insurance Company, including Senior Vice President and Comptroller, Vice President and Comptroller and Vice President-Internal Audit. Prior to joining Atlantic Mutual Insurance Company, Mr. Golding served as the Vice President of Ideal Mutual Insurance Company in 1979 and as the Assistant Controller of AIG, a position he held from December 1979 to March 1980. From 1974 to 1979 Mr. Golding served in various positions at Crum & Forster, including Assistant Controller and from 1972 to 1974 Mr. Golding was employed by the Robert Stigwood Organization. Prior to 1972, Mr. Golding worked for the independent accounting firm of Price Waterhouse (now PricewaterhouseCoopers) as an auditor. Mr. Golding serves on the Board of Directors of United Automobile Insurance Group, where he is a member of the Corporate Governance Committee, Audit Committee and Investment Committee, on the Board of Directors of Hudson City Bancorp, Inc., where he is Chairman of the Board's Risk Committee and a member of the Audit Committee and Nominating Committee, and as Trustee of the John A. Forster Trust. Mr. Golding previously served on the Board of Directors of Neurologix, Inc. where he was Chairman of the Audit Committee and a member of the Compensation Committee. Mr. Golding previously served on the Board of Directors of Somerset Hills Bancorp and National Atlantic Holding Corporation. Mr. Golding is a retired CPA and a member of the American Institute of CPAs and a member of the New York State Society of CPAs. A graduate of St. John Fisher College, Mr. Golding holds an MBA from Fairleigh Dickenson University. Mr. Golding is also a graduate of the Advanced Education Program at the Wharton School of the University of Pennsylvania.

JEFFREY PALEY, M.D. has served as a director of the Company since November 15, 2013. Since 2003, Dr. Paley has served as the founder of Access Medical Associates, PC, a clinical medical practice that provides comprehensive adult primary care services and acute care, and the founder and chief executive officer of Crimson Biomedical Consulting, a company which provides consulting services to financial institutions regarding healthcare investment decisions. Dr. Paley currently serves as a director of Kellbenx, a privately held, venture backed, biotechnology company. Dr. Paley holds a bachelor's degree in mathematics and a Rabbinic ordination from Yeshiva University and received his M.D. from Harvard Medical School. He completed his residency in internal medicine at Massachusetts

General Hospital Harvard Medical School. Dr. Paley was selected as a director because of his professional and medical experience.

Composition of the Board of Directors

Our bylaws provide that the size of our board of directors will be determined from time to time by resolution of our board of directors. Our board of directors currently consists of five directors, four of whom qualify as independent directors under the rules and regulations of the Securities and Exchange Commission, or SEC, and NASDAQ.

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Director Independence

Our securities are not listed on a national securities exchange or on any inter-dealer quotation system which has a requirement that a majority of directors be independent. We previously evaluated independence by the standards for director independence set forth in the NASDAQ Marketplace Rules.

Under these rules, a director is not considered to be independent if he or she is also an executive officer or employee of the corporation. As a result, Mr. Shkreli would not be considered independent because he serves as an executive officer of the Company. Our other directors, Messrs. Aselage, Golding, Paley and Richardson, would be considered independent under these rules.

Rule 5605 of the NASDAQ Marketplace Rules, or the NASDAQ Listing Rules, requires that independent directors compose a majority of a listed company's board of directors within one year of listing. In addition, the NASDAQ Listing Rules require that, subject to specified exceptions, each member of a listed company's audit, compensation, and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Exchange Act of 1934, as amended, or the Exchange Act. Under NASDAQ Listing Rule 5605(a)(2), a director will only qualify as an "independent director" if, in the opinion of our board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3 under the Exchange Act, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee: (1) accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries; or (2) be an affiliated person of the listed company or any of its subsidiaries. Beginning in 2014, in addition to satisfying general independence requirements under the NASDAQ Listing Rules, members of a compensation committee of a listed company must also satisfy additional independence requirements set forth in NASDAQ Listing Rule 5605(d)(2). In order to be considered independent for purposes of NASDAQ Listing Rule 5605(d)(2), a member of a compensation committee of a listed company may not, other than in his or her capacity as a member of the compensation committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries. Additionally, the board of directors of the listed company must consider whether the compensation committee member is an affiliated person of the listed company or any of its subsidiaries and, if so, must determine whether such affiliation would impair the director's judgment as a member of the compensation committee.

In December 2013, our board of directors undertook a review of the composition of our board of directors the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family and other relationships, including those relationships described under "Certain Relationships and Related Party Transactions," our board of directors determined that none of Messrs. Aselage, Golding, Paley and Richardson has a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and that each of these directors is "independent" as that term is defined under 5605(a)(2) of the NASDAQ Listing Rules. Mr. Shkreli is not considered independent because he currently serves as our chief executive officer. Our board of directors also determined that each member of the audit, compensation and talent development, and nominating and corporate governance committees satisfy the independence standards for such committees established by the SEC and the NASDAQ Listing Rules, as applicable. In making these determinations on the independence of our directors, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances our board of directors deemed relevant in determining independence, including the beneficial ownership of our capital stock by each non-employee director.

Board Leadership Structure

Our bylaws provide our board of directors with flexibility to combine or separate the positions of Chairman of the Board and Chief Executive Officer in accordance with its determination that utilizing one or the other structure would be in our best interests. At the current time, we do not have a Chairman of the Board. Our board of directors believes that oversight of our company is the responsibility of our board of directors as a whole, and this responsibility can be properly discharged without a Chairman. Our Chief Executive Officer, Mr. Shkreli, facilitates communications between members of our board of directors and works with our senior management in the preparation of the agenda

for each board meeting. All of our directors are encouraged to make suggestions for board of director's agenda items or pre-meeting materials.

Our board of directors has concluded that our current leadership structure is appropriate at this time. However, our board of directors will continue to periodically review our leadership structure and may make such changes in the future as it deems appropriate.

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Our independent directors will meet alone in executive session at no less than four regular meetings of our board of directors each year. Any of the non-employee members of our board may call additional executive sessions of the independent directors at any time, and any non-employee member of our board may call an executive session at the request of a majority of the independent directors. The purpose of these executive sessions is to promote open and candid discussion among non-employee directors.

Role of the Board in Risk Oversight

We face a number of risks, including those described under the caption “Risk Factors” contained elsewhere in this prospectus. Our board of directors believes that risk management is an important part of establishing, updating and executing on the company’s business strategy. Our board of directors, as a whole and at the committee level, has oversight responsibility relating to risks that could affect the corporate strategy, business objectives, compliance, operations, and the financial condition and performance of the company. Our board of directors focuses its oversight on the most significant risks facing the company and on its processes to identify, prioritize, assess, manage and mitigate those risks. Our board of directors and its committees receive regular reports from members of the company’s senior management on areas of material risk to the company, including strategic, operational, financial, legal and regulatory risks. While our board of directors has an oversight role, management is principally tasked with direct responsibility for management and assessment of risks and the implementation of processes and controls to mitigate their effects on the company.

The audit committee, as part of its responsibilities, oversees the management of financial risks, including accounting matters, liquidity and credit risks, corporate tax positions, insurance coverage, and cash investment strategy and results. The audit committee is also responsible for overseeing the management of risks relating to the performance of the company’s internal audit function, if required, and its independent registered public accounting firm, as well as our systems of internal controls and disclosure controls and procedures. The compensation and talent development committee is responsible for overseeing the management of risks relating to our executive compensation and overall compensation and benefit strategies, plans, arrangements, practices and policies. The nominating and corporate governance committee oversees the management of risks associated with our overall compliance and corporate governance practices, and the independence and composition of our board of directors. These committees provide regular reports, on at least a quarterly basis, to the full board of directors.

Committees of the Board

Immediately prior to our listing on The NASDAQ Global Market, our board of directors will have established a standing audit committee, compensation and talent development committee and nominating and corporate governance committee. The composition and responsibilities of each committee are described below. Members serve on these committees until their resignation or until otherwise determined by our board.

Audit Committee

The audit committee is responsible for assisting our board of directors in its oversight of the integrity of our financial statements, the qualifications and independence of our independent auditors, and our internal financial and accounting controls. The audit committee has direct responsibility for the appointment, compensation, retention (including termination) and oversight of our independent auditors, and our independent auditors report directly to the audit committee. The audit committee also prepares the audit committee report that the SEC requires to be included in our annual proxy statement.

The members of the audit committee are Messrs. Golding, Paley and Richardson, and Mr. Golding serves as chair of the audit committee. All members of the audit committee qualify as an independent director under the corporate governance standards of the NASDAQ Listing Rules and the independence requirements of Rule 10A-3 of the Exchange Act. Our board of directors has determined that Mr. Golding qualifies as an “audit committee financial expert” as such term is currently defined in Item 407(d)(5) of Regulation S-K. The audit committee has adopted a written charter that satisfies the applicable standards of the SEC and the NASDAQ Listing Rules, which we will post on our website upon completion of this offering.

Compensation and Talent Development Committee

The compensation and talent development committee approves the compensation objectives for the company, approves the compensation of the chief executive officer and approves or recommends to our board of directors for approval the compensation for other executives. The compensation and talent development committee reviews all

compensation components, including base salary, bonus, benefits and other perquisites.

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The members of the compensation and talent development committee are Messrs. Richardson, Aselage and Golding, and Mr. Richardson serves as chair of the compensation and talent development committee. Each member of the compensation and talent development committee is a non-employee director within the meaning of Rule 16b-3 of the rules promulgated under the Exchange Act, each is an outside director as defined by Section 162(m) of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and each is an independent director as defined by the NASDAQ Listing Rules, including NASDAQ Listing 5605(d)(2). The compensation and talent development committee has adopted a written charter that satisfies the applicable standards of the SEC and the NASDAQ Listing Rules, which we will post on our website upon completion of this offering.

Nominating and Corporate Governance Committee

The nominating and corporate governance committee is responsible for making recommendations to our board of directors regarding candidates for directorships and the structure and composition of our board and the board committees. In addition, the nominating and corporate governance committee is responsible for developing and recommending to our board corporate governance guidelines applicable to the company and advising our board on corporate governance matters.

The members of the nominating and corporate governance committee are Messrs. Aselage, Paley and Richardson, and Mr. Aselage serves as chair of the nominating and corporate governance committee. Each member of the nominating and corporate governance committee is a non-employee director within the meaning of Rule 16b-3 of the rules promulgated under the Exchange Act and an independent director as defined by the NASDAQ Listing Rules. The nominating and corporate governance committee has adopted a written charter that satisfies the applicable standards of the NASDAQ Listing Rules, which we will post on our website upon completion of this offering.

Code of Business Conduct and Ethics

We adopted a code of business conduct and ethics that applies to all of our employees, officers and directors including those officers responsible for financial reporting. Upon completion of this offering, we will post the code of business conduct and ethics on our website. We intend to disclose future amendments to the code or any waivers of its requirements on our website to the extent permitted by the applicable rules and exchange requirements.

Compensation Committee Interlocks and Insider Participation

None of the members of our compensation and talent development committee has ever been an officer or employee of the company. None of our executive officers serves, or has served during the last three year, as a member of the board of directors, compensation and talent development committee or other board committee performing equivalent functions of any entity that has one or more executive officers serving as one of our directors or on our compensation and talent development committee.

Compensation of Directors

For the fiscal year ended December 31, 2012, we did not pay any compensation to any of our non-employee directors. On December 6, 2013, our board of directors established a compensation policy for our non-employee directors pursuant to which each non-employee director shall receive $100,000 annually, which amount shall be comprised of not more than $25,000 in cash, with the remainder paid in the form of options to purchase shares of our common stock. Each non-employee director may, at his discretion, determine to receive less than $25,000 annually in the form of cash, in which case such amount will be paid to such director in the form of options to purchase additional shares of our common stock. In accordance with such policy, on December 6, 2013, each of Messrs. Aselage and Golding and Dr. Paley elected to receive $25,000 in cash and were accordingly each awarded options to purchase shares of our common stock at an exercise price of $8.70. Mr. Richardson elected to receive all of such compensation in the form of options, and accordingly was awarded options to purchase shares of our common stock at an exercise price of $8.70.

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EXECUTIVE COMPENSATION

The following table sets forth all cash compensation paid by the Company for the fiscal years 2012 and 2013. The table below sets forth the positions and compensation for each officer and director of the Company.

SUMMARY COMPENSATION TABLE

Name and Principal Position	Fiscal Year	Salary ($)	Bonus ($)	Stock Awards ($) (1)	Option Awards ($) (1)	All Other Compensation ($)	Total ($)
Martin Shkreli, Chief Executive Officer and Director (2)	2013	252,091	933,430	—	88,200 (5)	—	1,273,721
	2012	250,000	565,231	14,444,100	—	—	15,259,331
Marc Panoff, Chief Financial Officer	2013	142,153	27,488	76,800	—	—	246,441
	2012	—	—	—	—	—	—
Horacio Plotkin, Chief Medical Officer	2013	222,727	20,000	—	166,115	—	408,842
	2012	—	—	—	—	—	—
Stephen Aselage, Director (2) (3)	2013	—	—	—	—	—	—
	2012	83,333	—	2,000,000	—	—	2,083,333
Robert Wilson, former Chief Executive Officer, President and Director (4)	2013	—	—	—	—	—	—
	2012	—	—	—	—	—	—
Gary Lyons, former Director (4)	2013	—	—	—	—	—	—
	2012	—	—	—	—	—	—

(1)

- Amounts reflect the aggregate grant date fair value of stock awards computed in accordance with FASB ASC 718 and are not necessarily an indication of which named executive officers received the most gains from previously granted equity awards.

(2)

- The compensation data for Messrs. Shkreli and Aselage prior to December 12, 2012 reflects compensation paid by our predecessor, Retrophin, Inc., formerly known as Retrophin, LLC.

(3)

- Mr. Aselage’s compensation in respect of his service as a director of the Company in 2013 is set forth under “Director Compensation.”

(4)

- Prior to December 12, 2012, Robert Wilson served as the principal executive officer of Desert Gateway and, prior to December 17, 2012, Robert Wilson and Gary Lyons served as directors of Desert Gateway. Desert Gateway did not paid its officers and directors any salary or consulting fees in fiscal year 2012.

(5)

- The fair value of the 2013 option award to Mr. Shkreli reflected in the table above has been calculated assuming that such award has been outstanding since December 16, 2013, the grant date of such award.

Compensation Arrangements

Mr. Shkreli received an annual base salary of $250,000 pursuant to an employment agreement with our predecessor, former Retrophin. Mr. Shkreli received a bonus of $565,231 for fiscal 2012 and $933,430 for fiscal 2013. Mr. Shkreli's bonus for 2012 included a payment of $250,000 in connection with his performance. In addition to the $250,000 of base salary and $250,000 of performance bonus in 2012, the Company reclassified $407,900 in stockholders' equity as of December 31, 2012, of which approximately $93,000 related to expenses that should have been classified as operating expense. Of the remaining $315,231, we determined that our write-off in 2012 of a $210,000 note due from a related party that Mr. Shkreli managed prior to our merger with Desert Gateway should be deemed to be a bonus to him. Also, we determined that payments that we made prior to our merger with Desert Gateway in the amount of $105,231 were for operating expenses of the aforementioned related party and should be deemed to be a bonus to Mr. Shkreli. Mr. Shkreli did not receive direct payments in connection with the $315,000 in charges described in the prior two sentences, and elected for the Company to treat these payments as compensation.

While employed by former Retrophin, Mr. Shkreli received a total of 1,475,425 incentive units subject to vesting. Pursuant to Mr. Shkreli's award agreements, such unvested units immediately vested upon the Company's merger with Desert Gateway.

Grants of Stock Awards

On March 31, 2011, the Company granted 1,608,300 incentive shares to Mr. Shkreli, which vested on the final day of each calendar quarter over three years commencing on June 30, 2011. On September 11, 2012, the Company accelerated the vesting of 938.175 of the shares issued to Mr. Shkreli.

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In January 2012, the Company granted 801,600 incentive shares to Mr. Shkreli, which vested on the final day of each calendar quarter over three years, commencing on March 31, 2012. On September 11, 2012, the Company immediately vested Mr. Shkreli's 28,185 unvested incentive shares for continuing services. On December 11, 2012, Mr. Shkreli's 573,015 remaining unvested incentive shares were vested immediately in connection with the 2012 Merger.

All of such grants of incentive shares were originally issued as Class B incentive units of our predecessor, Retrophin, LLC, that represented a profits interest up through the date of former Retrophin's conversion to a C Corporation on September 20, 2012. Prior to former Retrophin's conversion to a corporation, shares granted as incentive shares were subject to certain conditions at the time of grant, which specified that the upon the occurrence of certain events, former Retrophin had the right to repurchase all vested incentive shares owned by such incentive shareholder. This repurchase option was rescinded upon former Retrophin's conversion to a corporation.

Employment Agreements

Shkreli Employment Agreement

On December 16, 2013, the Company entered into an employment agreement (the "Shkreli Employment Agreement") with Martin Shkreli, pursuant to which Mr. Shkreli will continue to serve as our Chief Executive Officer.

In accordance with the terms of the Shkreli Employment Agreement, Mr. Shkreli will be paid (i) a base salary in the amount of $300,000 (subject to adjustments at the discretion of the Board after each anniversary of the Effective Date), and (ii) at the sole discretion of the board, an annual bonus award based upon specific goals and performance metrics. Mr. Shkreli will also be awarded options to purchase 1,080,000 shares of restricted common stock of the Company, a pro rata portion of which will vest quarterly during the 3 years following the Effective Date. In the event of a change of control of the Company, all of Mr. Shkreli's unvested options shall immediately vest.

The Shkreli Employment Agreement contemplates that Mr. Shkreli's employment will be for a three-year term and may be automatically extended for successive three-year periods unless (i) Mr. Shkreli gives notice of non-extension to us no later than one hundred eighty (180) days prior to the expiration of the Agreement or (ii) Mr. Shkreli is terminated.

In the event Mr. Shkreli's employment is terminated by Mr. Shkreli for good reason (as such term is defined in the Shkreli Employment Agreement), then Mr. Shkreli will be entitled to continue to receive his annual base salary, any unpaid bonus and health insurance coverage on the same terms as made available to our employees for a period of twelve (12) months following such termination. If Mr. Shkreli's employment is terminated other than for good reason, Mr. Shrekli will forfeit any unvested stock options that he received and will not be entitled to severance or any additional payments.

If Mr. Shkreli's employment is terminated for cause (as such term is defined in the Shkreli employment Agreement) then Mr. Shkreli will not be entitled to any further payments of any kind, except for payment of base salary plus reimbursement of certain expenses.

In the event that Mr. Shkreli is no longer employed by us, any options that have not vested prior to the date of termination will be immediately cancelled and not subject to further vesting.

Plotkin Employment Agreement

On April 24, 2013, the Company entered into an employment agreement (the "Plotkin Employment Agreement") with Horacio Plotkin, M.D., pursuant to which Dr. Plotkin serves as the Chief Medical Officer of the Company starting on May 13, 2013.

In accordance with the terms of the Plotkin Employment Agreement, Dr. Plotkin will be paid (i) a base salary in the amount of $350,000 (subject to adjustments at the discretion of the Board after each anniversary of the Effective Date), and (ii) at the sole discretion of the board, an annual bonus award of up to 50% of Dr. Plotkin's then applicable base salary. Following the Effective Date, Dr. Plotkin will receive $20,000 in connection with signing the Plotkin Employment Agreement with the Company. Dr. Plotkin will also be awarded options to purchase 120,000 shares of restricted common stock of the Company, a pro rata portion of which will vest quarterly during the 3 years following the Effective Date.

The Plotkin Employment Agreement contemplates that Dr. Plotkin's employment will be for a one-year term and may be automatically extended for successive one-year periods unless (i) Dr. Plotkin gives notice of non-extension to the Company no later than ninety (90) days prior to the expiration of the Agreement, (ii) Dr. Plotkin is terminated or (iii)

the Company delivers notice to Dr. Plotkin no later than thirty (30) days prior to the expiration of the Agreement.
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In the event Dr. Plotkin's employment is terminated (i) by the Company without cause (as such term is defined in the Plotkin Employment Agreement) or (ii) by Dr. Plotkin's resignation following a material breach of a material term of the Plotkin Employment Agreement by the Company which has not been cured within 10 days following notice thereof, then Dr. Plotkin will be entitled to a severance payment in an amount equal to $60,000 plus reimbursement of certain expenses. If Dr. Plotkin chooses to resign for reasons other than a material breach of the Plotkin Employment Agreement by the Company then Dr. Plotkin will forfeit any unvested stock grants or stock options that he received and will not be entitled to severance or any additional payments.

If Dr. Plotkin's employment is terminated for cause then Dr. Plotkin will not be entitled to any further payments of any kind, except for payment of base salary plus reimbursement of certain expenses.

In the event that Dr. Plotkin is no longer employed by the Company, any options that have not vested prior to the date of termination will be immediately cancelled and not subject to further vesting.

Panoff Employment Agreement

On May 7, 2013, the Company entered into an employment agreement (the "Panoff Employment Agreement") with Marc Panoff, pursuant to which Mr. Panoff serves as the Chief Financial Officer and Chief Accounting Officer of the Company starting on May 20, 2013.

In accordance with the terms of the Panoff Employment Agreement, Mr. Panoff will be paid (i) a base salary in the amount of $230,000 (subject to adjustments at the discretion of the Board after each anniversary of the Effective Date), and (ii) at the sole discretion of the Board, an annual bonus award of up to 50% of Mr. Panoff s then applicable base salary. Mr. Panoff will also be granted 120,000 units of restricted common stock of the Company, a pro rata portion of which will vest quarterly during the 3 years following the execution of the Panoff Employment Agreement.

The Panoff Employment Agreement contemplates that Mr. Panoff's employment will be for a one-year term and may be automatically extended for successive one-year periods unless (i) Mr. Panoff gives notice of non-extension to the Company no later than ninety (90) days prior to the expiration of the Agreement, (ii) Mr. Panoff is terminated or (iii) the Company delivers notice to Mr. Panoff no later than thirty (30) days prior to the expiration of the Agreement.

In the event Mr. Panoff's employment is terminated (i) by the Company without cause (as such term is defined in the Panoff Employment Agreement) or (ii) by Mr. Panoff's resignation following a material breach of a material term of the Panoff Employment Agreement by the Company which has not been cured within 10 days following notice thereof, then Mr. Panoff will be entitled to a severance payment in an amount equal to $40,000 plus reimbursement of certain expenses. If Mr. Panoff chooses to resign for reasons other than a material breach of the Panoff Employment Agreement by the Company then Mr. Panoff will forfeit any unvested stock grants or stock options that he received and will not be entitled to severance or any additional payments.

If Mr. Panoff's employment is terminated for cause then Mr. Panoff will not be entitled to any further payments of any kind, except for payment of base salary plus reimbursement of certain expenses.

In the event that Mr. Panoff is no longer employed by the Company, any units of restricted stock that have not vested prior to the date of termination will be immediately cancelled and not subject to further vesting.

As of June 30, 2013, the Company and Mr. Panoff entered into an amendment to the Panoff Employment Agreement, pursuant to which the initial vesting of a pro rata portion of the restricted common stock granted under the Panoff Employment Agreement was changed from June 30, 2013 to December 31, 2013.

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OUTSTANDING EQUITY AWARDS AT FISCAL YEAR-END

The following table sets forth certain information with respect to the value of all equity awards that were outstanding at December 31, 2013 for each of our Named Executive Officers.

	Option Awards				Stock Awards	
Name	**Number of Securities Underlying Unexercised Options (#) Exercisable**	**Number of Securities Underlying Unexercised Options (#) Unexercisable**	**Option Exercise Price ($)**	**Option Expiration Date**	**Number of Shares or Units of Stock That Have Not Vested (#)**	**Market Value of Shares or Units of Stock That Have Not Vested ($)**
Martin Shkreli	90,000	990,000 (1)	$7.45	12/16/2023	—	—
Marc Panoff	—	—	—	—	108,000 (2)	$756,000
Horacio Plotkin	20,000	100,000 (3)	$8.70	5/13/2023	—	—

(1)

- Such options vest and become exercisable over a period of eleven calendar quarters beginning on March 31, 2014.

(2)

- Such restricted shares vest over a period of nine calendar quarters beginning March 31, 2014.

(3)

- Such options vest and become exercisable over a period of ten calendar quarters beginning on January 1, 2014.

DIRECTOR COMPENSATION

The following table summarizes the compensation we paid to our non-employee directors during the fiscal year ended December 31, 2013. Compensation information for Martin Shkreli, our Chief Executive Officer, is set forth in the Summary Compensation Table above.

Our policy is to pay non-employee directors $100,000 annually, which amount shall be comprised of not more than $25,000 in cash, with the remainder paid in the form of options to purchase shares of our common stock. Each non-employee director may, at his discretion, determine to receive less than $25,000 annually in the form of cash, in which case such amount will be paid to such director in the form of options to purchase additional shares of our common stock. Our director compensation policy was approved in the fourth quarter of 2013 and accordingly those directors electing to receive cash only received such cash for that quarter of service. The amounts below reflect the aggregate grant date fair value of stock awards computed in accordance with FASB ASC 718.

Name	Fees Earned or Paid in Cash ($)	Stock Awards ($)	Option Awards ($)	All Other Compensation ($)	Total ($)
Stephen Aselage	6,250	—	78,697	—	84,947
Cornelius E. Golding	6,250	—	78,697	—	84,947
Jeffrey Paley	6,250	—	78,697	—	84,947
Steven Richardson	—	—	98,372	—	98,372

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PRINCIPAL STOCKHOLDERS

The following table sets forth information known to us about the beneficial ownership of our common stock at January 9, 2014, as adjusted to reflect the sale of the shares of common stock in this offering, by:

- each of our named executive officers;
- each of our directors;
- each person known to us to be the beneficial owner of more than 5% of our common stock; and
- all of our executive officers and directors as a group.

Unless otherwise noted below, the address of each beneficial owner listed on the table is c/o Retrophin, Inc., 777 Third Avenue, 22nd Floor, New York, NY 10017. We have determined beneficial ownership in accordance with the rules of the SEC. Except as indicated by the footnotes below, we believe, based on the information furnished to us, that the persons and entities named in the table below have sole voting and investment power with respect to all shares of common stock that they beneficially own, subject to applicable community property laws. The table is based upon information supplied by officers, directors and principal stockholders and Schedules 13G or 13D filed with the Securities and Exchange Commission, or the SEC.

In computing the number of shares of common stock beneficially owned by a person and the percentage ownership of that person, we deemed outstanding shares of common stock subject to options or warrants held by that person that are currently exercisable or exercisable within 60 days of December 16, 2013. We did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person.

The percentages below prior to the offering are based on 18,376,363 shares of our common stock outstanding as of September 30, 2013. The percentages below after the offering are based on 23,082,245 shares of our common stock to be outstanding immediately after the completion of this offering, which gives effect to the issuance of 4,705,882 shares of common stock in this offering. Ownership information assumes no exercise by the underwriters of their option to purchase additional shares.

	Shares Beneficially Owned Prior to Offering				**Beneficial Ownership After Offering**		
Name of Beneficial Owner	**Shares**		**Percent**		**Shares**	**Percent**	
Executive Officers and Directors							
Martin Shkreli, Chief Executive Officer and Director	3,445,615	(1)	18.60	%	3,445,615	14.93	%
Marc Panoff, Chief Financial Officer	121,650	(2)	*		121,650	*	
Horatio Plotkin, M.D., Chief Medical Officer	33,300	(3)	*		33,300	*	
Stephen Aselage, Director	274,034	(4)	1.49	%	274,034	1.19	%
Steven Richardson, Director	117,620	(5)	*		117,620	*	
Cornelius E. Golding, Director	12,000	(6)	*		12,000	*	
Jeffrey Paley, M.D., Director	83,665	(7)	*		83,665	*	

	Shares Beneficially Owned Prior to Offering				Beneficial Ownership After Offering		
All directors and executive officers as a group	4,087,884		21.96	%	4,087,884	17.71	%
5% Stockholders							
Opaleye L.P.	2,139,941	(8)	11.08	%	2,139,941	9.27	%
Perceptive Life Sciences Master Fund Ltd	1,575,005	(9)	8.33	%	1,575,005	6.82	%
Sabby Healthcare Volatility Master Fund, Ltd.	1,297,945	(10)	6.97	%	1,297,945	5.62	%
QVT Fund V LP	1,285,857	(11)	6.84	%	1,285,857	5.57	%

*

- Less than 1%, unless otherwise specified.

(1)

- Includes an aggregate of 473,687 shares of common stock held by MSMB Healthcare LP and MSMB Healthcare Investors LLC. Mr. Shkreli is the managing member of MSMB Healthcare Investors LLC, which is the general partner of MSMB Healthcare LP. Mr. Shkreli disclaims beneficial ownership of the shares held by MSMB Healthcare LP and MSMB Healthcare Investors LLC. Includes 90,000 shares of common stock issuable upon exercise of stock options which have vested or will vest within 60 days of the date hereof. Includes 62,778 shares of common stock issuable upon exercise of warrants to purchase our common stock.

(2)

- Includes 550 shares of common stock issuable upon exercise of warrants to purchase our common stock.

(3)

- Includes 30,000 shares of common stock issuable upon exercise of stock options which have vested or will vest within 60 days of the date hereof. Includes 1,100 shares of common stock issuable upon exercise of warrants to purchase our common stock.

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(4)

- Includes 12,000 shares of common stock issuable upon exercise of stock options which have vested or will vest within 60 days of the date hereof. Includes 278 shares of common stock issuable upon exercise of warrants to purchase our common stock.

(5)

- Includes 15,000 shares of common stock issuable upon exercise of stock options which have vested or will vest within 60 days of the date hereof. Includes 555 shares of common stock issuable upon exercise of warrants to purchase our common stock.

(6)

- Includes 12,000 shares of common stock issuable upon exercise of stock options which have vested or will vest within 60 days of the date hereof.

(7)

- Includes 12,000 shares of common stock issuable upon exercise of stock options which have vested or will vest within 60 days of the date hereof.

(8)

- James Silverman shares voting and investment power with respect to the shares held by this stockholder. Such amount includes 938,441 shares of common stock issuable upon exercise of warrants to purchase our common stock.

(9)

- Joseph Edelman shares voting and investment power with respect to the shares held by this stockholder. Such amount includes 552,313 shares of common stock issuable upon exercise of warrants to purchase our common stock.

(10)

- Such amount includes (i) 1,047,945 shares of common stock and (ii) 250,000 shares of common stock issuable upon exercise of warrants to purchase our common stock. Sabby Management, LLC shares voting and investment power with respect to these shares on behalf of this stockholder. As manager of Sabby Management, LLC, Hal Mintz also shares voting and investment power on behalf of this stockholder. Each of Sabby Management, LLC and Hal Mintz disclaim beneficial ownership over the securities covered by this prospectus except to the extent of their pecuniary interest therein.

(11)

- Quintessence Fund L.P., QVT Fund IV LP, QVT Fund V LP and Fourth Avenue Capital Partners LP, together with their affiliates (and any other persons or entities whose beneficial ownership of common stock may be aggregated with them for purposes of Section 13(d) of the Exchange Act) (collectively, the “QVT Affiliates”), are collectively subject to a 9.99% beneficial ownership limitation with respect to the Warrants held by the QVT Affiliates. This beneficial ownership limitation prohibits the QVT Affiliates from acquiring shares of

common stock upon conversion of the warrants to purchase our common stock held by the QVT Affiliates to the extent that, upon such exercise, the number of shares of common stock then beneficially owned by the QVT Affiliates would exceed 9.99% of the total number of shares of common stock then issued and outstanding. As a result of such ownership limitation. Fourth Avenue Capital Partners LP is currently the beneficial owner of only 182,876 shares of common stock, consisting of (i) 144,446 shares of common stock and (ii) 38,430 warrants exercisable pursuant to the aforementioned beneficial ownership limitation.

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CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

2012 Merger

On December 12, 2012, in connection with the 2012 Merger, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement"), pursuant to which the Company acquired former Retrophin, our predecessor, in a transaction valued at approximately $13,585,300, based on the $2.50 closing price of our common stock on the OTC QB on such date.

Martin Shkreli. Prior to the 2012 Merger, Mr. Shkreli, our Chief Executive Officer and one of our directors, was the President and a director of our predecessor, and directly or indirectly held an aggregate of 611,384 vested and unvested shares, or approximately 65.31%, of the then outstanding shares of common stock, whether vested or unvested, and 46,241 shares, or approximately 29.74%, of the then outstanding shares of Series A Preferred Stock, of our predecessor. Mr. Shkreli obtained his current positions with the Company in connection with the 2012 Merger. In addition, as of the closing of the 2012 Merger, in which each share of common stock and Series A Preferred Stock of our predecessor were converted into five shares and seven shares, respectively, of our common stock, Mr. Shkreli became the beneficial owner, either directly or indirectly through entities controlled by him, of 3,380,607 shares of our common stock, with a value of approximately $8,451,518, based on the closing price of our common stock on the OTC QB on December 12, 2012, the date of the Merger Agreement for the 2012 Merger.

Stephen Aselage. Prior to the 2012 Merger, Mr. Aselage, one of our directors, held the same position with our predecessor, and held 50,000 vested and unvested shares, or approximately 5.34%, of the then outstanding shares of common stock, whether vested or unvested, and 1,600 shares, or approximately 1.03%, of the then outstanding shares of Series A Preferred Stock, of our predecessor. Mr. Aselage obtained his current position with the Company in connection with the 2012 Merger. In addition, as of the closing of the 2012 Merger, upon the conversion of his shares of our predecessor into shares of our common stock in accordance with the terms of the 2012 Merger, Mr. Aselage became the holder of 261,200 shares of our common stock, with a value of approximately $653,000, based on the closing price of our common stock on the OTC QB on December 12, 2012, the date of the Merger Agreement for the 2012 Merger.

Steven Richardson: Prior to the 2012 Merger, Mr. Richardson, one of our directors, was a director of our predecessor, and held 14,361 vested and unvested shares, or approximately 1.53%, of the then outstanding shares of common stock, whether vested or unvested, and 3,750 shares, or approximately 2.41%, of the then outstanding shares of Series A Preferred Stock, of our predecessor. Mr. Richardson obtained his current position with the Company in connection with the 2012 Merger. In addition, as of the closing of the 2012 Merger, upon the conversion of his shares of our predecessor into shares of our common stock in accordance with the terms of the 2012 Merger, Mr. Richardson became the holder of 98,055 shares of our common stock, with a value of approximately $245,138, based on the closing price of our common stock on the OTC QB on December 12, 2012, the date of the Merger Agreement for the 2012 Merger.

Private Placements

On February 14, 2013, the Company completed a private placement transaction (the "February 2013 Private Placement"), pursuant to a Securities Purchase Agreement, dated as of February 12, 2013, by and among the Company and certain purchasers, including Mr. Shkreli. In such private placement transaction, Mr. Shkreli purchased 120,000 shares of our common stock and Warrants to purchase up to 60,000 shares of our common stock, for an aggregate purchase price of $360,000. In connection with the closing of the February 2013 Private Placement, the Company also entered into a Registration Rights Agreement with such purchasers, including Mr. Shkreli, pursuant to which the Company agreed to register all of the shares of common stock, and shares of common stock issuable upon the exercise of the Warrants, sold in the February 2013 Private Placement.

On August 15, 2013, the Company completed a private placement transaction (the "August 2013 Private Placement"), pursuant to a Securities Purchase Agreement, dated as of August 14, 2013, by and among the Company and certain purchasers, including Mr. Shkreli, Mr. Panoff, Mr. Plotkin, Mr. Aselage and Mr. Richardson. In such private placement transaction, (i) Mr. Shkreli purchased 5,556 shares of our common stock and Warrants to purchase up to 2,778 shares of our common stock, for an aggregate purchase price of $25,000, (ii) Mr. Panoff purchased 1,100 shares of our common stock and Warrants to purchase up to 550 shares of our common stock, for an aggregate purchase price of $4,950, (iii) Mr. Plotkin purchased 2,200 shares of our common stock and Warrants to purchase up to 1,100

shares of our common stock, for an aggregate purchase price of $9,900, (iv) Mr. Aselage purchased 556 shares of our common stock and Warrants to purchase up to 278 shares of our common stock, for an aggregate purchase price of $2,500 and

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(v) Mr. Richardson purchased 1,110 shares of our common stock and Warrants to purchase up to 555 shares of our common stock, for an aggregate purchase price of $4,995. In connection with the closing of the August 2013 Private Placement, the Company also entered into a Registration Rights Agreement with such purchasers, including Mr. Shkreli, Mr. Panoff, Mr. Plotkin, Mr. Aselage and Mr. Richardson, pursuant to which the Company agreed to register all of the shares of common stock, and shares of common stock issuable upon the exercise of the Warrants, sold in the August 2013 Private Placement.

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DESCRIPTION OF CAPITAL STOCK

The following summary describes our capital stock and the material provisions of our certificate of incorporation and our bylaws. Because the following is only a summary, it does not contain all of the information that may be important to you. For a complete description, you should refer to our certificate of incorporation and bylaws, copies of which have been filed as exhibits to the registration statement of which this prospectus is part.

General

We currently have authorized capital stock of 120,000,000 shares, of which 100,000,000 shares are designated as common stock, par value $0.0001 per share, and 20,000,000 shares are designated as preferred stock, par value $0.0001 per share, of which 1,000 shares are designated as Class A Preferred, par value $0.001 per share. As of September 30, 2013, 18,376,363 shares of our common stock and 0 shares of our preferred stock were issued and outstanding. As of September 30, 2013, there were 290 holders of record of our common stock.

Common Stock

The holders of our common stock are entitled to one vote per share on matters on which our stockholders vote. There are no cumulative voting rights. Subject to any preferential dividend rights of any outstanding shares of preferred stock, holders of our common stock are entitled to receive dividends, if declared by our board of directors, out of funds that we may legally use to pay dividends. Generally, all matters to be voted on by stockholders must be approved by a majority (or, in the case of election of directors, by a plurality) of the votes entitled to be cast by all shares of our Common Stock that are present in person or represented by proxy.

Holders representing fifty percent (50%) of our Common Stock issued, outstanding and entitled to vote, represented in person or by proxy, are necessary to constitute a quorum at any meeting of our stockholders. A vote by the holders of a majority of our outstanding shares is required to effectuate certain fundamental corporate changes such as liquidation, merger or an amendment to our Articles of Incorporation. If we liquidate or dissolve, holders of our common stock are entitled to share ratably in our assets once our debts and any liquidation preference owed to any then-outstanding preferred stockholders are paid. Our certificate of incorporation does not provide our common stock with any redemption, conversion or preemptive rights.

Preferred Stock

Our board of directors has the authority, without further action by our stockholders, to issue up to 20,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting any series or the designation of such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change of control of our company or other corporate action. Upon consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Warrants

As of September 30, 2013, we had outstanding warrants to purchase up to an aggregate of 4,462,876 shares of common stock.

Registration Rights

We are party to a pair of registration rights agreements. The first is a registration rights agreement, as amended, with the purchasers in the February Private Placement. The second is a registration rights agreement with the purchasers in the August Private Placement.

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February 2013 Registration Rights Agreement, as Amended

On February 14, 2013, in connection with the closing of the February Private Placement, we entered into a Registration Rights Agreement with the purchasers in the February Private Placement, which sets forth the rights of such purchasers to have their shares of common stock purchased in the February Private Placement and shares of common stock issuable upon exercise of the Warrants registered with the SEC for public resale. On December 6, 2013, the SEC declared effective the Registration Statement covering the shares of common stock purchased in the February Private Placement and shares of common stock issuable upon exercise of the Warrants.

We have agreed to use reasonable efforts to maintain the effectiveness of such Registration Statement until all of the securities covered by such Registration Statement have or may be sold by investors under Rule 144 of the Securities Act, without volume or manner-of-sale restrictions. If we fail to maintain the effectiveness of such Registration Statement as required for specified time periods, we are required pay to the holders of registrable securities, on the date of each such event and on each monthly anniversary thereof until the applicable event is cured, partial liquidated damages equal to 2.0% of the aggregate purchase price paid by such purchaser in the February Private Placement, up to a maximum of 10.0% of such aggregate purchase price. If we fail to pay any partial liquidated damages pursuant to this section in full within seven days after the date payable, we will pay interest thereon at a rate of 18% per annum (or such lesser maximum amount that is permitted to be paid by applicable law) to such purchaser, accruing daily from the date such partial liquidated damages are due until such amounts, plus all such interest thereon, are paid in full.

August 2013 Registration Rights Agreement

On August 15, 2013, in connection with the closing of the August 15, 2013 private placement (the “August Private Placement”), We entered into a Registration Rights Agreement with the purchasers in the August Private Placement, which sets forth the rights of such purchasers to have their shares of common stock purchased in the August Private Placement and shares of common stock issuable upon exercise of the Warrants registered with the SEC for public resale. On December 6, 2013, the SEC declared effective the Registration Statement covering the shares of common stock purchased in the August Private Placement and shares of common stock issuable upon exercise of the Warrants.

We have agreed to use reasonable efforts to maintain the effectiveness of such Registration Statement until all of the securities covered by such Registration Statement have or may be sold by investors under Rule 144 of the Securities Act, without volume or manner-of-sale restrictions. If we fail to maintain the effectiveness of such Registration Statement as required for specified time periods, we are required pay to the holders of registrable securities, on the date of each such event and on each monthly anniversary thereof until the applicable event is cured, partial liquidated damages equal to 2.0% of the aggregate purchase price paid by such purchaser in the August Private Placement, up to a maximum of 10.0% of such aggregate purchase price. If we fail to pay any partial liquidated damages pursuant to this section in full within seven days after the date payable, we will pay interest thereon at a rate of 18% per annum (or such lesser maximum amount that is permitted to be paid by applicable law) to such purchaser, accruing daily from the date such partial liquidated damages are due until such amounts, plus all such interest thereon, are paid in full.

Anti-Takeover Effects of Provisions of our Certificate of Incorporation, our Bylaws and Delaware Law

Some provisions of Delaware law, our certificate of incorporation and our bylaws contain provisions that could make the following transactions more difficult: acquisition of us by means of a tender offer; acquisition of us by means of a proxy contest or otherwise; or removal of our incumbent officers and directors. It is possible that these provisions could make it more difficult to accomplish or could deter transactions that stockholders may otherwise consider to be in their best interest or in our best interests, including transactions that might result in a premium over the market price for our shares.

These provisions, summarized below, are expected to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors. We believe that the benefits of increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure us outweigh the disadvantages of discouraging these proposals because negotiation of these proposals could result in an improvement of their terms.

Preferred Stock

Our board of directors has the authority, without further action by our stockholders, to issue up to 20,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. The issuance of preferred stock could have the effect of delaying, deferring or preventing a change of control of our company or other corporate action.

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Delaware Anti-Takeover Statute

We are subject to Section 203 of the Delaware General Corporation Law, which prohibits persons deemed "interested stockholders" from engaging in a "business combination" with a publicly-held Delaware corporation for three years following the date these persons become interested stockholders unless the business combination is, or the transaction in which the person became an interested stockholder was, approved in a prescribed manner or another prescribed exception applies. Generally, an "interested stockholder" is a person who, together with affiliates and associates, owns, or within three years prior to the determination of interested stockholder status did own, 15% or more of a corporation's voting stock. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the interested stockholder. The existence of this provision may have an anti-takeover effect with respect to transactions not approved in advance by the board of directors, such as discouraging takeover attempts that might result in a premium over the market price of our common stock.

Exchange Listing

Prior to this offering, our common stock was not listed on any national securities exchange and was quoted on the OTC QB market under the symbol "RTRX." In connection with this offering, our common stock has been approved for listing on The NASDAQ Global Market under the trading symbol "RTRX".

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Standard Registrar & Transfer Co. Inc. The transfer agent and registrar's address is 12528 South 1840 East, Draper, UT 84020, and its telephone number is (801) 571-8844.

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Underwriting

Subject to the terms and conditions set forth in the underwriting agreement, dated January 9, 2014, between us and Jefferies LLC, as the representative of the underwriters named below and the sole book-running manager of this offering, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the respective number of shares of common stock shown opposite its name below:

Underwriter	Number of Shares
Jefferies LLC	3,294,117
Roth Capital Partners, LLC	658,823
Ladenburg Thalmann & Co. Inc.	376,471
Summer Street Research Partners	376,471
Total	4,705,882

The underwriting agreement provides that the obligations of the several underwriters are subject to certain conditions precedent such as the receipt by the underwriters of officers' certificates and legal opinions and approval of certain legal matters by their counsel. The underwriting agreement provides that the underwriters will purchase all of the shares of common stock if any of them are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the nondefaulting underwriters may be increased or the underwriting agreement may be terminated. We have agreed to indemnify the underwriters and certain of their controlling persons against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that the underwriters may be required to make in respect of those liabilities.

The underwriters have advised us that, following the completion of this offering, they currently intend to make a market in the common stock as permitted by applicable laws and regulations. However, the underwriters are not obligated to do so, and the underwriters may discontinue any market-making activities at any time without notice in their sole discretion. Accordingly, no assurance can be given as to the liquidity of the trading market for the common stock, that you will be able to sell any of the common stock held by you at a particular time or that the prices that you receive when you sell will be favorable.

The underwriters are offering the shares of common stock subject to their acceptance of the shares of common stock from us and subject to prior sale. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

Commission and Expenses

The underwriters have advised us that they propose to offer the shares of common stock to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers, which may include the underwriters, at that price less a concession not in excess of $0.3315 per share of common stock. After the offering, the initial public offering price, concession and reallowance to dealers may be reduced by the representative. No such reduction will change the amount of proceeds to be received by us as set forth on the cover page of this prospectus.

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The following table shows the public offering price, the underwriting discounts and commissions that we are to pay the underwriters and the proceeds, before expenses, to us in connection with this offering. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Per Share		Total	
	Without Option to Purchase Additional Shares	With Option to Purchase Additional Shares	Without Option to Purchase Additional Shares	With Option to Purchase Additional Shares
Public offering price	$8.5000	$ 8.5000	$39,999,997	$ 45,999,994
Underwriting discounts and commissions paid by us	$0.5525	$ 0.5525	$2,600,000	$ 2,990,000
Proceeds to us, before expenses	$7.9475	$ 7.9475	$37,399,997	$ 43,009,994

We estimate expenses payable by us in connection with this offering, other than the underwriting discounts and commissions referred to above, will be approximately $829,000 . We have also agreed to reimburse the underwriters for up to $30,000 for their FINRA counsel fee. In accordance with FINRA Rule 5110, this reimbursed fee is deemed underwriting compensation for this offering.

In a private placement that closed on August 15, 2013, we sold 5,531,401 shares of our common stock at a purchase price of $4.50 a share, or $24,891,304 in the aggregate, and warrants to purchase up to an aggregate of 2,765,701 shares of our common stock with an exercise price of $6.00 per share underlying each warrant. Three employees of Roth Capital, LLC, a co-managing underwriter for this offering, each acquired 2,303 shares of common stock and 1,111 warrants in this private placement. In accordance with FINRA Rule 5110, the securities acquired by such employees of Roth Capital, LLC are deemed to constitute an aggregate of $41,010 of underwriting compensation in connection with this offering.

Listing

Our common stock has been approved for listing on The NASDAQ Global Market under the trading symbol "RTRX".

Stamp Taxes

If you purchase shares of common stock offered in this prospectus, you may be required to pay stamp taxes and other charges under the laws and practices of the country of purchase, in addition to the offering price listed on the cover page of this prospectus.

Option to Purchase Additional Shares

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase, from time to time, in whole or in part, up to an aggregate of 705,882 shares from us at the public offering price set forth on the cover page of this prospectus, less underwriting discounts and commissions. If the underwriters exercise this option, each underwriter will be obligated, subject to specified conditions, to purchase a number of additional shares proportionate to that underwriter's initial purchase commitment as indicated in the table above. This option may be exercised only (i) if the underwriters sell more shares than the total number set forth on the cover page of this prospectus and (ii) with our prior written consent.

No Sales of Similar Securities

We, our officers, directors and holders of all or substantially all our outstanding capital stock and other securities have agreed, subject to specified exceptions, not to directly or indirectly:

- sell, offer, contract or grant any option to sell (including any short sale), pledge, transfer, establish an open "put equivalent position" within the meaning of Rule 16a-l(h) under the Securities Exchange Act of 1934, as amended, or

- otherwise dispose of any shares of common stock, options or warrants to acquire shares of common stock, or securities exchangeable or exercisable for or convertible into shares of common stock currently or hereafter owned either of record or beneficially, or

- publicly announce an intention to do any of the foregoing for a period of 90 days after the date of this prospectus without the prior written consent of Jefferies LLC.

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This restriction terminates after the close of trading of the common stock on and including the 90 th day after the date of this prospectus.

Jefferies LLC may, in its sole discretion and at any time or from time to time before the termination of the 90-day period release all or any portion of the securities subject to lock-up agreements. There are no existing agreements between the underwriters and any of our shareholders who will execute a lock-up agreement, providing consent to the sale of shares prior to the expiration of the lock-up period.

Stabilization

The underwriters have advised us that they, pursuant to Regulation M under the Securities Exchange Act of 1934, as amended, and certain persons participating in the offering may engage in short sale transactions, stabilizing transactions, syndicate covering transactions or the imposition of penalty bids in connection with this offering. These activities may have the effect of stabilizing or maintaining the market price of the common stock at a level above that which might otherwise prevail in the open market. Establishing short sales positions may involve either "covered" short sales or "naked" short sales.

"Covered" short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares of our common stock in this offering. The underwriters may close out any covered short position by either exercising their option to purchase additional shares of our common stock or purchasing shares of our common stock in the open market. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the option to purchase additional shares.

"Naked" short sales are sales in excess of the option to purchase additional shares of our common stock. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the shares of our common stock in the open market after pricing that could adversely affect investors who purchase in this offering.

A stabilizing bid is a bid for the purchase of shares of common stock on behalf of the underwriters for the purpose of fixing or maintaining the price of the common stock. A syndicate covering transaction is the bid for or the purchase of shares of common stock on behalf of the underwriters to reduce a short position incurred by the underwriters in connection with the offering. Similar to other purchase transactions, the underwriter's purchases to cover the syndicate short sales may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock may be higher than the price that might otherwise exist in the open market. A penalty bid is an arrangement permitting the underwriters to reclaim the selling concession otherwise accruing to a syndicate member in connection with the offering if the common stock originally sold by such syndicate member are purchased in a syndicate covering transaction and therefore have not been effectively placed by such syndicate member.

Neither we, nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our common stock. The underwriters are not obligated to engage in these activities and, if commenced, any of the activities may be discontinued at any time.

The underwriters may also engage in passive market making transactions in our common stock on The NASDAQ Global Market in accordance with Rule 103 of Regulation M during a period before the commencement of offers or sales of shares of our common stock in this offering and extending through the completion of distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded.

Electronic Distribution

A prospectus in electronic format may be made available by e-mail or on the web sites or through online services maintained by one or more of the underwriters or their affiliates. In those cases, prospective investors may view offering terms online and may be allowed to place orders online. The underwriters may agree with us to allocate a specific number of shares of common stock for sale to online brokerage account holders. Any such allocation for online distributions will be made by the underwriters on the same basis as other allocations. Other than the prospectus in electronic format, the information on the underwriters' web sites and any information contained in any other web

site maintained by any of the underwriters is not part of this prospectus, has not been approved and/or endorsed by us or the underwriters and should not be relied upon by investors.

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Other Activities and Relationships

The underwriters and certain of their affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. The underwriters and certain of their affiliates have, from time to time, performed, and may in the future perform, various commercial and investment banking and financial advisory services for us and our affiliates, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and certain of their affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers, and such investment and securities activities may involve securities and/or instruments issued by us and our affiliates. If the underwriters or their respective affiliates have a lending relationship with us, they routinely hedge their credit exposure to us consistent with their customary risk management policies. The underwriters and their respective affiliates may hedge such exposure by entering into transactions which consist of either the purchase of credit default swaps or the creation of short positions in our securities or the securities of our affiliates, including potentially the common stock offered hereby. Any such short positions could adversely affect future trading prices of the common stock offered hereby. The underwriters and certain of their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

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NOTICE TO INVESTORS

Australia

This prospectus is not a disclosure document for the purposes of Australia's Corporations Act 2001 (Cth) of Australia, or Corporations Act, has not been lodged with the Australian Securities & Investments Commission and is only directed to the categories of exempt persons set out below. Accordingly, if you receive this prospectus in Australia: You confirm and warrant that you are either:

- a "sophisticated investor" under section 708(8)(a) or (b) of the Corporations Act;
- a "sophisticated investor" under section 708(8)(c) or (d) of the Corporations Act and that you have provided an accountant's certificate to the company which complies with the requirements of section 708(8)(c)(i) or (ii) of the Corporations Act and related regulations before the offer has been made; or
- a "professional investor" within the meaning of section 708(11)(a) or (b) of the Corporations Act.

To the extent that you are unable to confirm or warrant that you are an exempt sophisticated investor or professional investor under the Corporations Act any offer made to you under this prospectus is void and incapable of acceptance. You warrant and agree that you will not offer any of the shares issued to you pursuant to this prospectus for resale in Australia within 12 months of those shares being issued unless any such resale offer is exempt from the requirement to issue a disclosure document under section 708 of the Corporations Act.

European Economic Area

In relation to each member state of the European Economic Area which has implemented the Prospectus Directive, each referred to herein as a Relevant Member State, with effect from and including the date on which the Prospectus Directive is implemented in that Relevant Member State, referred to herein as the Relevant Implementation Date, no offer of any securities which are the subject of the offering contemplated by this prospectus has been or will be made to the public in that Relevant Member State other than any offer where a prospectus has been or will be published in relation to such securities that has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the relevant competent authority in that Relevant Member State in accordance with the Prospectus Directive, except that with effect from and including the Relevant Implementation Date, an offer of such securities may be made to the public in that Relevant Member State:

- to any legal entity which is a "qualified investor" as defined in the Prospectus Directive;
- to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives of the underwriters for any such offer; or
- in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of securities shall require the Company or any of the underwriters to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe the securities, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Member State and the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

Hong Kong

No securities have been offered or sold, and no securities may be offered or sold, in Hong Kong, by means of any document, other than to persons whose ordinary business is to buy or sell shares or debentures, whether as principal or agent; or to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any

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rules made under that Ordinance; or in other circumstances which do not result in the document being a "prospectus" as defined in the Companies Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap.32) of Hong Kong. No document, invitation or advertisement relating to the securities has been issued or may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted under the securities laws of Hong Kong) other than with respect to securities which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance.

This prospectus has not been registered with the Registrar of Companies in Hong Kong. Accordingly, this prospectus may not be issued, circulated or distributed in Hong Kong, and the securities may not be offered for subscription to members of the public in Hong Kong. Each person acquiring the securities will be required, and is deemed by the acquisition of the securities, to confirm that he is aware of the restriction on offers of the securities described in this prospectus and the relevant offering documents and that he is not acquiring, and has not been offered any securities in circumstances that contravene any such restrictions.

Japan

The offering has not been and will not be registered under the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948 of Japan, as amended), or FIEL, and the Initial Purchaser will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means, unless otherwise provided herein, any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the FIEL and any other applicable laws, regulations and ministerial guidelines of Japan.

Singapore

This prospectus has not been and will not be lodged or registered with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or the invitation for subscription or purchase of the securities may not be issued, circulated or distributed, nor may the securities be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to the public or any member of the public in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA, (ii) to a relevant person as defined under Section 275(2), or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions, specified in Section 275 of the SFA, or (iii) otherwise pursuant to, and in accordance with the conditions of any other applicable provision of the SFA.

Where the securities are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- a corporation (which is not an accredited investor as defined under Section 4A of the SFA) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

- a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary is an accredited investor,

shares, debentures and units of shares and debentures of that corporation or the beneficiaries' rights and interest in that trust shall not be transferable for six months after that corporation or that trust has acquired the Offer Shares under Section 275 of the SFA except:

- to an institutional investor under Section 274 of the SFA or to a relevant person defined in Section 275(2) of the SFA, or to any person pursuant to an offer that is made on terms that such shares, debentures and units of shares and debentures of that corporation or such rights and interest in that trust are acquired at a consideration of not less than $200,000 (or its equivalent in a foreign currency) for each transaction, whether such amount is to be paid for in cash or by exchange of securities or other assets, and further for corporations, in accordance with the conditions, specified in Section 275 of the SFA;

- where no consideration is given for the transfer; or

- where the transfer is by operation of law.

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Switzerland

The securities may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This prospectus has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this prospectus nor any other offering or marketing material relating to the securities or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this prospectus nor any other offering or marketing material relating to the offering, the Company or the securities have been or will be filed with or approved by any Swiss regulatory authority. In particular, this prospectus will not be filed with, and the offer of securities will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of securities has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of securities.

United Kingdom

This prospectus is only being distributed to, and is only directed at, persons in the United Kingdom that are qualified investors within the meaning of Article 2(1)(e) of the Prospectus Directive that are also (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended, referred to herein as the Order, and/or (ii) high net worth entities falling within Article 49(2)(a) to (d) of the Order and other persons to whom it may lawfully be communicated. Each such person is referred to herein as a Relevant Person.

This prospectus and its contents are confidential and should not be distributed, published or reproduced (in whole or in part) or disclosed by recipients to any other persons in the United Kingdom. Any person in the United Kingdom that is not a Relevant Person should not act or rely on this document or any of its contents.

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MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following summary describes the material U.S. federal income and estate tax consequences of the acquisition, ownership and disposition of our common stock acquired in this offering by Non-U.S. Holders (as defined below). This discussion does not address all aspects of U.S. federal income and estate taxes and does not deal with foreign, state and local consequences that may be relevant to Non-U.S. Holders in light of their particular circumstances, nor does it address U.S. federal tax consequences other than income and estate taxes. Special rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Code such as financial institutions, insurance companies, tax-exempt organizations, broker-dealers and traders in securities, U.S. expatriates, "controlled foreign corporations," "passive foreign investment companies," corporations that accumulate earnings to avoid U.S. federal income tax, persons that hold our common stock as part of a "straddle," "hedge," "conversion transaction," "synthetic security" or integrated investment or other risk reduction strategy, partnerships and other pass-through entities, and investors in such pass-through entities. Such Non-U.S. Holders are urged to consult their own tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them. Furthermore, the discussion below is based upon the provisions of the Code, and Treasury regulations, rulings and judicial decisions thereunder as of the date hereof, and such authorities may be repealed, revoked or modified, perhaps retroactively, so as to result in U.S. federal income and estate tax consequences different from those discussed below. We have not requested a ruling from the U.S. Internal Revenue Service, or IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions. This discussion assumes that the Non-U.S. Holder holds our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment).

The following discussion is for general information only and is not tax advice. Persons considering the purchase of our common stock pursuant to this offering should consult their own tax advisors concerning the U.S. federal income and estate tax consequences of acquiring, owning and disposing of our common stock in light of their particular situations as well as any consequences arising under the laws of any other taxing jurisdiction, including any state, local or foreign tax consequences.

For the purposes of this discussion, a "Non-U.S. Holder" is, for U.S. federal income tax purposes, a beneficial owner of common stock that is neither a U.S. Holder, a partnership (or other entity treated as a partnership for U.S. federal income tax purposes regardless of its place of organization or formation), nor an entity that is treated as a disregarded entity for U.S. federal income tax purposes (regardless of its place of organization or formation). A "U.S. Holder" means a beneficial owner of our common stock that is for U.S. federal income tax purposes (a) an individual who is a citizen or resident of the United States, (b) a corporation or other entity treated as a corporation created or organized in or under the laws of the United States, any state thereof or the District of Columbia, (c) an estate the income of which is subject to U.S. federal income taxation regardless of its source or (d) a trust if it (1) is subject to the primary supervision of a court within the United States and one or more U.S. persons have the authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable U.S. Treasury regulations to be treated as a U.S. person.

The discussion below assumes that a Non-U.S. Holder's investment in our common stock is not effectively connected with a trade or business conducted in the United States by a Non-U.S. holder or, if a tax treaty applies to the Non-U.S. Holder, that its investment is not attributable to a United States permanent establishment maintained by the Non-U.S. Holder.

Distributions

Although we do not anticipate that we will make any distributions on our common stock in the foreseeable future, to the extent we make distributions on our common stock out of our current or accumulated earnings and profits (as determined under U.S. federal income tax principles), such distributions will constitute dividends for U.S. tax purposes and will be subject to U.S. withholding tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. To obtain a reduced rate of withholding under a treaty, a Non-U.S. Holder generally will be required to provide us with a properly executed IRS Form W-8BEN, or successor form, certifying the Non-U.S. Holder's entitlement to benefits under that treaty. In the case of a Non-U.S. Holder that is an entity, Treasury regulations and the relevant tax treaty provide rules to determine whether, for purposes of determining the applicability of a tax treaty, dividends will be treated as paid to the entity or to those holding an interest in that entity.

If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the holder's behalf, the holder will be required to provide appropriate documentation to such agent. The holder's agent will then be required to provide certification to us or our paying agent, either directly or through other

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intermediaries. If a Non-U.S. Holder is eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty, the Non-U.S. Holder should contact its tax advisor regarding the possibility of obtaining a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

To the extent distributions on our common stock, if any, exceed our current and accumulated earnings and profits, they will constitute a non-taxable return of capital and will first reduce the Non-U.S. Holder's adjusted basis in our common stock, but not below zero, and then will be treated as gain and taxed in the same manner as gain realized from a sale or other disposition of common stock as described in the next section. If it cannot be determined at the time a distribution is made whether the distribution will exceed our current and accumulated earnings and profits, then the distribution will be subject to withholding at the rate applicable to ordinary dividends. However, a Non-U.S. Holder may seek a refund of these amounts from the IRS if it is subsequently determined that the distribution did, in fact, exceed our current and accumulated earnings and profits.

Gain on Disposition of Our Common Stock

Subject to the discussion below, a Non-U.S. Holder generally will not be subject to U.S. federal income tax with respect to gain realized on a sale or other disposition of our common stock unless (a) the Non-U.S. Holder is a nonresident alien individual and is present in the United States for 183 or more days in the taxable year of the disposition and certain other conditions are met, or (b) we are or have been a "United States real property holding corporation" within the meaning of Code Section 897(c)(2) at any time within the shorter of the five-year period preceding such disposition or such holder's holding period. In general, we would be a United States real property holding corporation if interests in U.S. real estate comprised (by fair market value) at least half of our business assets. We believe that we are not, and do not anticipate becoming, a United States real property holding corporation. Even if we are treated as a United States real property holding corporation, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the Non-U.S. Holder owned, directly, indirectly and constructively, no more than five percent of our common stock at all times within the shorter of (i) the five-year period preceding the disposition or (ii) the holder's holding period and (2) our common stock is regularly traded on an established securities market. There can be no assurance that our common stock will qualify as regularly traded on an established securities market.

If you are an individual Non-U.S. Holder described in (a) above, you will be required to pay a flat 30% tax on the gain derived from the sale, which gain may be offset by certain U.S. source capital losses (even though you are not considered a resident of the United States). Non-U.S. Holders should consult any applicable income tax or other treaties that may provide for different rules.

Information Reporting Requirements and Backup Withholding

Generally, we must report information to the IRS with respect to any dividends we pay on our common stock including the amount of any such dividends, the name and address of the recipient, and the amount, if any, of tax withheld. A similar report is sent to the holder to whom any such dividends are paid. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities in the recipient's country of residence. Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding. U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN or otherwise establishes an exemption. The current backup withholding rate is 28%.

Under current U.S. federal income tax law, U.S. information reporting and backup withholding requirements generally will apply to the proceeds of a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or foreign, except that information reporting and such requirements may be avoided if the holder provides a properly executed IRS Form W-8BEN or otherwise meets documentary evidence requirements for establishing Non-U.S. Holder status or otherwise establishes an exemption. Generally, U.S. information reporting and backup withholding requirements will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a non-U.S. broker. Information reporting and backup withholding requirements may, however, apply to a payment of disposition proceeds if the broker has actual knowledge, or reason to know, that the holder is, in fact, a U.S. person. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers.

Backup withholding is not an additional tax. A holder subject to backup withholding should contact the holder's tax advisor regarding the possibility of obtaining a refund or a tax credit and any associated requirements to provide information to the IRS or other relevant tax authority.

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FATCA Withholding

The Foreign Account Tax Compliance Act, or FATCA, imposes a 30% withholding tax on certain types of payments made to "foreign financial institutions" and certain other non-U.S. entities unless certain due diligence, reporting, withholding, and certification requirements are satisfied.

As a general matter, FATCA imposes a 30% withholding tax on dividends on, and gross proceeds from the sale or other disposition of, our common stock if paid to a foreign entity unless either (i) the foreign entity is a "foreign financial institution" that undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) the foreign entity is not a "foreign financial institution" and identifies certain of its U.S. investors, or (iii) the foreign entity otherwise is excepted under FATCA.

Pursuant to the delayed effective dates provided for in the final regulations, the required withholding does not begin until July 1, 2014, with respect to dividends on our common stock and January 1, 2017, with respect to gross proceeds from a sale or other disposition of our common stock.

If withholding is required under FATCA on a payment related to our common stock, investors that otherwise would not be subject to withholding (or that otherwise would be entitled to a reduced rate of withholding) generally will be required to seek a refund or credit from the IRS to obtain the benefit of such exemption or reduction (provided that such benefit is available). Prospective investors should consult their tax advisors regarding the effect of FATCA in their particular circumstances.

Federal Estate Tax

An individual Non-U.S. Holder who is treated as the owner of, or has made certain lifetime transfers of, an interest in our common stock will be required to include the value thereof in his or her gross estate for U.S. federal estate tax purposes, and may be subject to U.S. federal estate tax unless an applicable estate tax treaty provides otherwise, even though such individual was not a citizen or resident of the United States at the time of his or her death.

THE PRECEDING DISCUSSION OF U.S. FEDERAL INCOME AND ESTATE TAX CONSIDERATIONS IS FOR GENERAL INFORMATION ONLY. IT IS NOT TAX ADVICE. EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW.

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LEGAL MATTERS

The validity of our common stock offered hereby will be passed upon by Katten Muchin Rosenman LLP, New York, New York. Covington & Burling LLP, New York, New York, is counsel to the underwriters in connection with this offering.

EXPERTS

The audited consolidated financial statements of Retrophin, Inc., our predecessor, and its subsidiaries as of December 31, 2012 and 2011 and the related statements of operations, changes in stockholder's deficiency and cash flows for the year ended December 31, 2012 and the periods from March 11, 2011 (inception) through December 31, 2012 and March 11, 2011 (inception) through December 31, 2011, and related footnotes appearing in this registration statement, have been so included in reliance on the report of Marcum LLP, an independent registered public accounting firm, appearing elsewhere in this prospectus, given on the authority of such firm as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We file reports and other information with the SEC. We have also filed a registration statement on Form S-1, including exhibits, with the SEC with respect to the shares being offered in this offering. This prospectus is part of the registration statement, but it does not contain all of the information included in the registration statement or exhibits. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference. You may inspect any materials we file with the SEC at the SEC's Public Reference Room at 100 F Street NE, Washington, D.C 20549, on any business day during the hours of 10:00 am to 3:00 pm. and copies of any such materials may be obtained from the SEC upon payment of the prescribed fee You may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. The SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding registrants that file electronically with the SEC. The address of the Internet site is http://www.sec.gov.

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(A DEVELOPMENT STAGE COMPANY)
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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONDENSED CONSOLIDATED BALANCE SHEETS

	September 30, 2013 (unaudited)	December 31, 2012
Assets		
Current assets:		
Cash	$13,409,825	$11,388
Marketable securities, available-for-sale	2,957,376	—
Prepaid expenses and other current assets	480,647	21,830
Total current assets	16,847,848	33,218
Property and equipment, net	38,437	23,790
Patents pending	23,793	18,093
Due from affiliate	—	137,547
Security deposits	177,547	—
Deposits on license agreements	2,250,000	—
Technology license, net	2,027,085	2,178,617
Total assets	$21,364,710	$2,358,047
Liabilities and Stockholders' Deficit		
Current liabilities:		
Technology license liability	$—	$1,300,000
Accounts payable	1,721,511	1,023,320
Accrued expenses	1,511,848	2,467,796
Settlements payable	1,691,400	—
Note payable—related party	—	884,764
Investors’ deposits	—	100,000
Due to related parties	10,000	23,200
Derivative financial instruments, at estimated fair value—warrants	22,234,325	—
Total current liabilities	27,169,084	5,799,080
Stockholders’ Deficit:		
Preferred stock Series A $0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 18,376,363 and 8,952,905 issued and outstanding, respectively	1,838	895
Additional paid-in capital	48,649,970	30,203,402
Deficit accumulated during the development stage	(54,301,348)	(33,612,112)
Accumulated other comprehensive income	(154,834)	—
Total stockholders’ deficit	(5,804,374)	(3,407,815)
Total liabilities and stockholders' deficit	$21,364,710	$2,391,265

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	For the three months ended September 30,		For the nine months ended September 30,		For the period from March 11, 2011 (inception) through September 30, 2013
	2013	2012	2013	2012	
Operating expenses:					
Compensation and related costs— inclusive of share base compensation $28,519, $4,780,383, $70,189, $7,724,150 and $17,808,002	$478,741	$5,068,707	$1,767,195	$8,371,481	$22,127,948
Professional fees—inclusive of share based compensation $1,640,501, $2,247,292, $1,849,745, $6,290,252 and $8,501,449	2,463,804	3,167,242	4,392,673	7,761,899	13,602,012
Research and development—inclusive of share based compensation $72,888, $0, $109,571, $0 and $109,571	1,399,875	110,656	2,113,813	286,889	3,008,326
Selling, general and administrative	812,066	113,140	4,131,193	337,622	5,487,301
Technology license fee	—	—	100,000	—	1,800,000
Total operating expenses	5,154,486	8,459,745	12,504,874	16,757,891	46,025,587
Operating loss	(5,154,486)	(8,459,745)	(12,504,874)	(16,757,891)	(46,025,587)
Other income (expense):					
Interest income	4	6,049	9	15,781	21,914
Interest expense	—	(26,761)	(41,563)	(70,559)	(147,480)
Registration payment obligation income	360,000	—	360,000	—	360,000
Registration payment obligation expense	(360,000)	—	(360,000)	—	(360,000)
Realized gain on sale of marketable securities	59,737	—	59,737	—	59,737
Change in fair value of derivative financial instruments—warrants	(5,803,054)	—	(8,198,672)	—	(8,198,672)

	For the three months ended September 30,		For the nine months ended September 30,		For the period from March 11, 2011 (inception) through September 30, 2011
Loss on transactions denominated in foreign currencies	—	—	(3,873)	—	(11,260)
Total other expense, net	(5,743,313)	(20,712)	(8,184,362)	(54,778)	(54,301,348)
Net loss	$(10,897,799)	$(8,480,457)	$(20,689,236)	$(16,812,669)	$(54,301,348)
Net loss per common share, basic and diluted	$(0.71)	$(2.42)	$(1.62)	$(5.55)	
Weighted average common shares outstanding, basic and diluted	15,365,631	3,510,415	12,797,714	3,027,468	
Comprehensive Loss:					
Net loss	$(10,897,799)	$(8,480,457)	$(20,689,236)	$(16,812,669)	$(54,301,348)
Unrealized loss on marketable securities	(154,834)	—	(154,834)	—	(154,834)
Comprehensive Loss	$(11,052,633)	$(8,480,457)	$(20,844,070)	$(16,812,669)	$(54,456,182)

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONDENSED CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' DEFICIT
FOR THE PERIOD FROM MARCH 11, 2011 (INCEPTION) THROUGH SEPTEMBER 30, 2013

	Common stock		Additional paid in capital	Receivables due from stockholder	Accumulated other comprehensive loss	Accumulated deficit	Total Stockholde deficit
	Shares	Amount					
ance—March 11, 2011 eption)	—	$—	$—	$—	$—	$—	$—
ance of common es	1,608,300	161	24,839	(25,000)	—	—	—
ance of common es to founders in nection with the al capital tribution	50,000	5	95	—	—	—	100
ntive shares ted—employees	1,758,300	176	(176)	—	—	—	—
ntive shares ted—non employees	381,000	38	(38)	—	—	—	—
ntive shares eited—employees	(45,835)	(5)	5	—	—	—	—
re based pensation—employees	—	—	1,724,967	—	—	—	1,724,967
re based pensation—non loyees	—	—	254,332	—	—	—	254,332
ance of shares in nection with ch 2011 private ement, net of fees of ,061	253,750	25	658,914	—	—	—	658,939
ance of Series A erred in connection March 2011 private ement, net of fees of 67, recapitalization ommon stock n made to kholder	36,750	4	103,629	—	—	—	103,633
loss	—	—	—	(10,000)	—	—	(10,000
ance—December 31, 1	—	—	—	—	—	(3,268,256)	(3,268,256
r Issuance of Series referred in nection with uary 2012 private ement, net of fees of	4,042,265	404	2,766,567	(35,000)	—	(3,268,256)	(536,285
	326,963	33	1,806,644	—	—	—	1,806,677

	Common stock		Additional paid in capital	Receivables due from stockholder	Accumulated other comprehensive loss	Accumulated deficit	Total Stockholde deficit
,677, exchanged to mon stock							
r Issuance of Series referred in nection with 2012 private ement, net of fees of ,275, exchanged to mon stock	470,764	47	1,668,979	—	—	—	1,669,026
res transferred to sultants by founder services	—	—	4,400,000	—	—	—	4,400,000
res transferred to loyees by founders services	—	—	1,375,000	—	—	—	1,375,000
res issued in ordance with license ement	620,000	62	1,549,938	—	—	—	1,550,000
res outstanding at e of reverse merger December 12, 2012	2,585,583	259	1,142	—	—	—	1,401
ntive shares ted—employees	866,180	86	(86)	—	—	—	—
ntive shares ted—non employees	87,503	9	(9)	—	—	—	—
ntive shares eited—employees	(46,353)	(5)	5	—	—	—	—
re based pensation—employees	—	—	14,637,850	—	—	—	14,637,850
re based pensation—non loyees	—	—	1,997,372	—	—	—	1,997,372
eivable due from kholder charged to pensation	—	—	—	407,900	—	—	407,900
n made to kholder	—	—	—	(372,900)	—	—	(372,900
loss	—	—	—	—	—	(30,343,856)	(30,343,856

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONDENSED CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' DEFICIT
FOR THE PERIOD FROM MARCH 11, 2011 (INCEPTION) THROUGH SEPTEMBER 30, 2013 (continued)

	Common stock		Additional paid in capital	Receivables due from stockholder	Accumulated other comprehensive loss	Accumulated deficit	Total Stockhold deficit
	Shares	Amount					
nce—December 31,	8,952,905	$895	$30,203,402	$—	$—	$(33,612,112)	$(3,407,815
tive shares ed—employees udited)	135,000	14	(14)	—	—	—	—
e based pensation—consultants udited)	194,000	19	1,282,201	—	—	—	1,282,220
e based pensation—employees udited)	—	—	179,760	—	—	—	179,760
e based pensation—non oyees (unaudited)	—	—	567,525	—	—	—	567,525
tive shares ited—employees udited)	(20,833)	(2)	2	—	—	—	—
tive shares ited—non employees udited)	(37,500)	(4)	4	—	—	—	—
nce of common in connection with ary 2013 private ement at $3.00 per , net of fees of $0 udited)	272,221	27	816,637	—	—	—	816,664
nce of common in connection with uary 2013 private ement at $3.00 per , net of fees of ,986 and registration ment obligation of ,000 (unaudited)	3,045,929	305	3,592,891	—	—	—	3,593,196
nce of common in connection with ust 2013 private ement at $4.50 per , net of fees of 1,313 and payment to February	5,531,401	553	10,639,270	—	—	—	10,639,823

	Common stock		Additional paid in capital	Receivables due from stockholder	Accumulated other comprehensive loss	Accumulated deficit	Total Stockhold deficit
tors for inducement rticipate in August cing of $2,238,681 udited)							
nce of common in connection with ent made to uary investors for cement to participate ugust financing, 222 shares at $4.50 hare and 20,685 s at $5.00 per share udited)	291,907	29	1,323,894	—	—	—	1,323,923
es issued on behalf ated party	6,000	1	44,399	—	—	—	44,400
stment to existing holder (unaudited)	5,333	1	(1)	—	—	—	—
alized loss on etable securities	—	—	—	—	(154,834)	—	(154,834
oss (unaudited)	—	—	—	—	—	(20,689,236)	(20,689,23
nce—September 30, (unaudited)	18,376,363	$1,838	$48,649,970	$—	$(154,834)	$(54,301,348)	$(5,804,374

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

	For the nine months ended September 30,		For the period from March 11, 2011 (inception) through September 30, 2013
	2013	2012	
Cash Flows From Operating Activities:			
Net loss	$(20,689,236)	$ (16,812,669)	$(54,301,348)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	159,128	73,417	284,368
Gain on securities available for sale	(59,737)	—	(59,737)
Compensation in lieu of receivable	—	—	407,900
Share based compensation—consultants	1,282,220	—	1,282,220
Share based compensation—employees	179,760	7,724,150	17,917,577
Share based compensation—non-employees	567,525	6,290,252	7,219,229
Registration payment obligation expense	360,000	—	—
Reversal of registration payment obligation liability	(360,000)	—	—
Share based payment—Technology license contingent fee	—	—	1,550,000
Change in estimated fair value of derivative financial instruments—warrants	8,198,672	—	8,198,672
Changes in operating assets and liabilities:			
Prepaid expenses	(458,817)	(30,431)	(480,647)
Other assets	—	(8,781)	—
Technology license fees	—	—	150,000
Settlement payable	1,691,400	—	1,691,400
Accounts payable and accrued expenses	(313,357)	675,251	3,175,438
Net cash used in operating activities	(9,442,442)	(2,088,811)	(12,964,928)
Cash Flows From Investing Activities:			
Purchase of fixed assets	(22,243)	(8,471)	(49,889)
Purchase of intangible assets	(5,700)	(1,158,418)	(1,173,793)
Payments for security deposits for exclusivity of certain licenses	(2,250,000)	—	(2,250,000)
Increase in security deposit	(40,000)	—	(40,000)
Repayment of technology license liability	(1,300,000)	—	(1,300,000)
Purchase of marketable securities, available-for-sale	(3,430,418)	—	(3,430,418)
Proceeds from the sale of marketable securities, available-for-sale	377,945	—	377,945
Cash received in merger transaction	—	—	3,721
Due from related parties	—	(2,800)	—
Payments made on behalf of affiliate	—	—	(137,547)

	For the nine months ended September 30,		For the period from March 11, 2011 (inception) through September 30, 2013
Loans made to stockholder	—	(399,329)	—
Net cash used in investing activities	(6,670,416)	(1,569,018)	(8,239,434)
Cash Flows From Financing Activities:			
Proceeds from related parties	—	—	56,500
Repayment of net amounts due to related parties	(13,200)	(30,000)	(56,700)
Proceeds from note payable—related party	—	930,000	930,000
Repayment of note payable—related party	(884,764)	(15,236)	(930,000)
Investors' deposits	—	—	100,000
Proceeds received from issuance of common stock, net	31,355,455	2,765,201	35,593,830
Payment to investors participated in August 2013 financing	(946,196)	—	(946,196)
Net cash provided by financing activities	29,511,295	3,649,965	34,757,634
Net increase in cash	13,398,437	(7,864)	13,409,825
Cash, beginning of period	11,388	10,053	—
Cash, end of period	$13,409,825	$ 2,189	$13,409,825

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (continued)

	For the nine months ended September 30,		For the period from March 11, 2011 (inception) through September 30, 2013
	2013	2012	
Supplemental Disclosure of Cash Flow Information:			
Cash paid for interest	$28,263	$ 9,764	$43,027
Non-cash investing and financing activities:			
Unrealized loss on marketable securities	$(154,834)	$ —	$(154,834)
Forfeiture of subscription receivable	$—	$ —	$25,000
Reclassification of due from related parties	$—	$ 500	$—
Technology license liability	$—	$ 1,300,000	$—
Shares issued on behalf of related party	$44,400	$ —	$44,400
Affiliate receivable applied to security deposit	$137,547	$ —	$137,547

The accompanying notes are an integral part of these condensed consolidated financial statements.
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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1. DESCRIPTION OF BUSINESS

Organization and Description of Business

Retrophin, Inc. (the "Company") is an emerging biotechnology company dedicated to developing drugs for rare and life-threatening diseases. The Company's primary business objective is to develop and commercialize therapies for orphan diseases, such as Duchenne muscular dystrophy, or DMD, focal segmental glomerulosclerosis, and pantothenate kinase-associated neurodegeneration. The Company is considered to be a development stage company and, as such, the Company's financial statements are prepared in accordance with the Accounting Standards Codification ("ASC") 915 "Development Stage Entities." The Company is subject to all of the risks and uncertainties associated with development stage companies.

NOTE 2. BASIS OF PRESENTATION

The accompanying unaudited condensed consolidated financial statements of the Company 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/A for the year ended December 31, 2012 (the "2012 10-K/A") filed with the Securities and Exchange Commission (the "SEC") on September 13, 2013. The accompanying condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("U.S. 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 U.S. 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 results for a full year. The December 31, 2012 balance sheet information was derived from the audited financial statements as of that date.

NOTE 3. LIQUIDITY, FINANCIAL CONDITION AND MANAGEMENT PLANS

The Company incurred a net loss of approximately $54 million, including stock-based compensation charge of approximately $26 million for the period from March 11, 2011 (inception) to September 30, 2013. At September 30, 2013, the Company had a cash balance of approximately $13,500,000 and a working capital deficit of approximately $10 million; however, the working capital deficit includes a derivative liability of approximately $22.2 million for warrants issued in financing transactions. The Company's accumulated deficit amounted to approximately $54 million at September 30, 2013.

The Company has principally financed its operations from inception using proceeds from sales of its equity securities in a series of private placement transactions (see Note 11). The Company to date has no revenues, significantly limited capital resources and is subject to all of the risks and uncertainties that are typical of a development stage enterprise. Significant uncertainties include, among others, whether it will be able to raise the additional capital it needs to finance the start of its planned operations and whether such operations, if launched, will enable the Company to become a profitable enterprise.

On August 14, 2013, the Company and the investors who participated in the private placement transaction that the Company completed on February 14, 2013, entered into the first amendment to the registration rights agreement (the "Amended Registration Rights Agreement") associated with that transaction. The Amended Registration Rights Agreement provides, among other things, for (i) a waiver of any and all liquidated damages that the Company incurred for its inability to cause a registration statement to be declared effective within certain contractually defined time-frames stipulated in the original agreement; (ii) a commitment on the part of the investors in the February private placement to participate in a private placement transaction that the Company completed on August 15, 2013, and (iii) a covenant on the part of the Company to proceed with the sale of shares that were issued under the August 15, 2013 private placement transaction. In exchange, the Company paid an aggregate fee to these investors of $2,495,256 consisting of (i) 73,710 shares of the Company's common stock with an aggregate fair value of $331,695 (based on the selling price of $4.50 per share in the August financing transaction); (ii) cash in the amount of $1,835,000; and (iii) warrants to purchase 98,756 shares of common stock with a fair value of $328,561. The investors were also given the

option to purchase shares of the Company’s
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common stock at $4.50 as a use of the cash portion of the payment arrangement. Accordingly, $946,196 of the cash portion of the fee was settled in cash and the remainder was settled by the issuance of 197,512 shares. Additionally, the Company paid $103,425 to an investor to whom the Company sold shares in a private placement transaction in January 2013 and who participated in the August 2013 private placement transaction. This payment was settled entirely by the issuance of 20,685 shares of the Company's common stock at a value of $5.00 per share (see Note 11).

On August 16, 2013, the Company announced that it had signed an agreement with a major pharmaceutical company for the exclusive right to negotiate a royalty-bearing U.S. license for a product to be developed for the treatment of Autism and Schizophrenia. Pursuant to the exclusivity agreement, the Company paid the major pharmaceutical company a non-refundable upfront fee of $2 million and will have an exclusive period of 120 days to negotiate a license agreement (see Note 13). The Company is in active negotiations to consummate a license agreement. If a definitive license agreement is consummated, the Company will apply the upfront fee to the license and will make a determination as to whether it should be treated as an asset or research and development expense. If no definitive agreement is consummated, the upfront fee will be expensed.

On September 20, 2013, the Company signed an agreement with an individual for the exclusive right to negotiate a royalty-bearing U.S. license for a product to be developed for the treatment of central nervous system disorders. Pursuant to the exclusivity agreement, the Company paid the individual a non-refundable upfront fee of $250,000 and will have an exclusive period ending on December 31, 2013 (see Note 13). The Company is in active negotiations to consummate a license agreement. If a definitive license agreement is consummated, the Company will apply the upfront fee to the license and will make a determination as to whether it should be treated as an asset or research and development expense. If no definitive agreement is consummated, the upfront fee will be expensed.

Effective October 1, 2013, the Company signed a Sponsored Research Agreement ("SRA") with St. Jude Children's Research Hospital ("St. Jude"). Unless otherwise terminated by operation of law or by acts of the parties in accordance with the terms of the agreement, the SRA shall be in full force and effect for a period of two (2) years and shall expire on October 1, 2015. The term may be extended by written agreement between the parties. The Company and St. Jude will collaborate on research focused on the study of PKAN and other infectious diseases (see Note 13 and Note 14).

In the second quarter of 2013, the Company, its Chief Executive Officer and a related party became parties to a series of agreements to settle up to $2,284,511 of liabilities, which Company management believes are the primary obligation of the related party. The Company paid $593,111 of these settlements in the second quarter on behalf of the related party and had outstanding liabilities of $1,691,400 as of September 30, 2013, which the Company paid as of the date of this filing. Concurrent with the execution and payment of such settlement agreements, the Company entered into indemnification agreements and received promissory notes from the related party whereby the related party agreed to pay the Company the principal amount of $2,284,511 plus interest at an annualized rate of 5% as reimbursement of payments that the Company made to settle a portion of the agreements. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the third quarter ended September 30, 2013. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary (see Note 10).

In addition, on August 29, 2013, the Company entered into and paid an additional settlement agreement for $300,000.

On September 18, 2013, the Company made a proposal to the board of directors of Transcept Pharmaceuticals, Inc. ("Transcept") to acquire all of the outstanding shares of Transcept's common stock for $4.00 per share in cash. The proposal has been rejected by Transcept's board of directors. The Company has invested approximately $3 million and acquired approximately 4.45% of the outstanding common stock of Transcept as part of the proposal process. If Transcept accepts the Company's proposal to acquire all of its outstanding shares of common stock, the Company will need to obtain additional equity or debt financing to consummate the acquisition and consolidation (see Note 14).

Management believes the Company's ability to continue its operations depends on its ability to raise capital. The Company's future depends on the costs, timing, and outcome of regulatory reviews of its product candidates and the costs of commercialization activities, including product marketing, sales and distribution. During the first quarter of 2013, the Company raised an aggregate of approximately $9.95 million in certain private placement transactions. During the third quarter of 2013, the Company raised an additional $24.92 million in aggregate proceeds in

connection with a private placement transaction. The Company expects to continue to finance its cash needs through additional private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups,

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foundations and government agencies. Although management believes that the Company has access to capital resources, there are no commitments for financing in place at this time, nor can management provide any assurance that such financing will be available on commercially acceptable terms, if at all.

These conditions raise substantial doubt about the Company's ability to continue as a going concern. These unaudited condensed consolidated financial statements do not include any adjustments relating to the recovery of assets or the classification of liabilities that might be necessary should the Company be unable to continue as a going concern.

NOTE 4. 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 subsidiary in conformity with U.S. GAAP. All intercompany accounts and transactions have been eliminated in consolidation.

Cash

For purposes of the statement of cash flows, the Company considers cash instruments with maturities of less than three months when purchased to be cash equivalents. There are no cash equivalents as of the balance sheet dates.

Marketable Securities

The Company accounts for marketable securities held as "available-for-sale" pursuant to ASC 320 Investments—Debt and Equity Securities ("ASC 320"). The Company classifies these investments as current assets and carry them at fair value. Unrealized gains and losses are recorded as a separate component of stockholders' equity as accumulated other comprehensive income. Realized gains or losses on marketable security transactions are reported in earnings and computed using the specific identification of cost basis. Marketable securities are maintained at one financial institution and are governed by the Company's investment policy as approved by our Board of Directors. Fair values of marketable securities are based on quoted market prices. Valuation of marketable securities are further describe in Note 5.

The Company's current investment policy generally limits security investments for purposes of strategic acquisitions. Based on the liquidity position of the Company, the CEO and CFO are authorized to make various investment transaction decisions for prudent investment of the Company's excess funds. The ability to conduct investments is limited to the CEO and CFO. The current policy limit marketable securities investments with a maturity, credit quality, and concentration that is authorized only by the CEO and CFO.

Employee Stock-Based Compensation

The Company accounts for stock-based compensation in accordance with ASC 718 Compensation—Stock Compensation ("ASC 718"). ASC 718 addresses all forms of share-based payment ("SBP") awards including shares issued under employee stock purchase plans and stock incentive shares. Under ASC 718 awards result in a cost that is measured at fair value on the awards' grant date, based on the estimated number of awards that are expected to vest and will result in a charge to operations.

Non-Employee Stock-Based Compensation

The Company accounts for equity instruments issued to non-employees in accordance with the provisions of ASC 505, "Equity Based Payments to Non-Employees", ("ASC 505") and ASC 718 which requires that such equity instruments are recorded at their fair value on the measurement date. The measurement of stock-based compensation is subject to periodic adjustment as the underlying equity instruments vest. Non-employee stock-based compensation charges are being amortized over their respective contractual vesting periods.

Use of Estimates

In preparing financial statements in conformity with U.S. GAAP, management is required to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting period. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions. These estimates and

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assumptions include valuing equity securities in share-based payments, estimating fair value of equity instruments recorded as derivative liabilities, estimating the useful lives of depreciable and amortizable assets and estimating the fair value of long-lived assets to assets whether impairment charges may apply.

Research and Development Costs

Research and development costs are charged to operations as incurred and consist primarily of consulting costs, contract research and development costs, and compensation costs. For the three and nine months ended September 30, 2013 and 2012, and for the period from March 11, 2011 (inception) through September 30, 2013, the Company recognized $1,399,875, $110,656, $2,113,813, $286,889, and $3,008,326, respectively, of research and development costs.

Patents

The Company capitalized external cost, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company expense cost associated with maintaining and defending patents subsequent to their issuance in the period incurred. The Company amortizes patent cost once issued on a straight-line basis over the estimate useful lives of the patents. The Company assess the potential impairment to all capitalized patent cost when events or changes in circumstances indicate that the carrying amount of our patent may not be recoverable. The Company accounts for patent costs in accordance with ASC Topic 350, “Goodwill and Other Intangible Assets” (“ASC 350”) and ASC Topic 805, “Business Combinations” (“ASC 805”).

Basic and diluted Net Loss Per Share

Basic and diluted net loss per share has been computed by dividing net loss by the weighted average number of common shares outstanding during the period. All potentially dilutive common shares have been excluded since their inclusion would be anti-dilutive.

An aggregate of 4,462,426 and 0 warrants were excluded from the computation of diluted net loss per common share for the three and nine months ended September 30, 2013 and 2012 because their inclusion would have an anti-dilutive effect for the periods presented.

An aggregate of 210,000 and 0 stock options were excluded from the computation of diluted net loss per common share for the three and nine months ended September 30, 2013 and 2012 because they would have an anti-dilutive effect for the periods presented.

An aggregate of 211,073 and 927,310 incentive shares were excluded from the computation of diluted net loss per common share for the three and nine months ended September 30, 2013 and 2012 because they were contingent shares subject to recall.

Derivative Instruments

The Company does not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. The Company evaluates all of its financial instruments to determine if such instruments are derivatives or contain features that qualify as embedded derivatives. For derivative financial instruments that are accounted for as liabilities, the derivative instrument is initially recorded at its fair value and is then revalued at each reporting date, with changes in the fair value reported in the statements of operations. For stock-based derivative financial instruments, the Company calculates the fair value of the financial instruments using a probability-weighted Black-Scholes option pricing model, which is comparable to the Binomial Lattice options pricing model at inception and on each subsequent valuation date. The classification of derivative instruments, including whether such instruments should be recorded as liabilities or as equity, is evaluated at the end of each reporting period (see Note 6 and Note 7).

Joint and Several Liability Assessment

The Company measures obligations resulting from joint and several liability arrangements as the sum of the amount that the Company has a) contractually agreed to pay, and b) any additional amounts that the Company expects to pay on behalf of its co-obligors.

Financial Instruments and Fair Value

ASC Topic 820, “Fair Value Measurements and Disclosures,” (“ASC Topic 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 Topic 820 are described below:

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

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

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

In estimating the fair value of the Company's marketable securities available-for-sale, the Company used quoted prices in active markets (see Note 5 and Note 7).

In estimating the fair value of the Company's derivative liabilities, the Company used a probability-weighted Black-Scholes option pricing model (see Note 6 and Note 7).

Financial assets with carrying values approximating fair value include cash as well as marketable securities, deposits on license agreements, prepaid expenses and other current assets. Financial liabilities with carrying values approximating fair value include accounts payable and accrued expenses.

Registration Payment Arrangement

The Company accounted for registration rights agreements in accordance with ASC 825-20, "Registration Payment Arrangements." ASC 825-20 addresses an issuer's accounting for registration payment arrangements. This pronouncement specifies that the contingent obligation to make future payments or otherwise transfer consideration under a registration payment arrangement, whether issued as a separate agreement or included as a provision of a financial instrument, should be separately recognized and accounted for as a contingency in accordance with ASC 450-20 "Loss Contingencies".

Reclassifications

Certain prior year financial statement balances have been reclassified to conform to the current year presentation. These reclassifications had no effect on the recorded net loss.

Subsequent Events

The Company follows the provisions of ASC Topic 855-10, "Subsequent Events," relating to subsequent events. This guidance establishes principles and requirements for subsequent events. This guidance defines the period after the balance sheet date during which events or transactions that may occur would be required to be disclosed in a company's financial statements. The Company has evaluated subsequent events up to the date of issuance of this report.

Recently Issued Accounting Pronouncements

In February 2013, the FASB issued Accounting Standards Updated ("ASU") 2013-04 "Obligations Resulting from Joint and Several Liability Arrangements for Which the Amount at the Reporting Date is Fixed") ("ASU 2013-04"). The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount the entity has a) contractually agreed to pay, and b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company early adopted this guidance in the second quarter of 2013 (see Note 3 and Note 10).

Except as noted above, management does not believe that any recently issued, but not yet effective accounting pronouncements, if adopted, would have a significant effect on the accompanying consolidated financial statements.

NOTE 5. MARKETABLE SECURITIES

The Company measures marketable securities on a recurring basis. Generally, the types of securities the Company invests in are traded on a market such as the NASDAQ Global Market, which the Company considers to be Level 1 inputs.

Marketable securities at September 30, 2013 consisted of the following:

	Cost	Unrealized Gains	Unrealized Losses	Estimated Fair Value
Marketable securities available-for-sale:	$3,112,210	$—	$154,834	$2,957,376

The Company’s marketable securities are comprised entirely of the common stock of Transcept.

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NOTE 6. DERIVATIVE FINANCIAL INSTRUMENTS

In accordance with ASC Topic 815-40, "Derivative and Hedging—Contracts in Entity's Own Equity" ("ASC Topic 815-40"), instruments which do not have fixed settlement provisions are deemed to be derivative instruments. Based upon the Company's analysis of the criteria contained in ASC Topic 815-40, the warrants issued in connection with the sale of the common stock during the period ended September 30, 2013 that do not have fixed settlement provisions, are not indexed to Company's own stock. The fair value of the warrants are classified as derivative liabilities due to a ratchet provision that allows for a favorable adjustment to the exercise price if the Company issues additional equity 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 valuation adjustments within other income (expense) in the Company's results of operations. The Company recorded a loss on a change in the estimated fair value of warrants of $5,803,054 and $8,198,672 for the three and nine months ended September 30, 2013, respectively.

The Company calculated the fair value of the warrants using a probability-weighted Black-Scholes option pricing model which is comparable to the Binomial Lattice pricing model. The assumptions used at the date of issuance and at September 30, 2013 are noted in the following table:

		As of		
	Date of issuance February 14, 2013	**Date of issuance August 14, 2013**	**Date of issuance August 15, 2013**	**September 30, 2013**
Fair market price of common stock	$3.75	$4.50	$4.50	$6.50
Contractual term	5 years	5 years	5 years	4.38-4.87 years
Risk-free interest rate	0.86%	1.48%	1.48%	1.41%
Expected volatility	101%	106%	106%	96%-102%

Expected volatility is based on historical stock volatilities of several comparable publicly-traded companies over a period equal to the expected terms of the warrants, as the Company does not have a long trading history to estimate the volatility of its own common stock. The warrants have a transferability provision. Based on guidance provided in SEC Staff Accounting Bulletin No. 107 ("SAB 107") for options issued with such a provision, the Company used the full contractual term as the initial expected term of the warrants. 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 7. FAIR VALUE MEASUREMENTS

The following table presents the Company's asset and liability that is measured and recognized at fair value on a recurring basis classified under the appropriate level of the fair value hierarchy as of September 30, 2013:

		Fair Value Measurements at September 30, 2013		
	Total carrying value at September 30, 2013	**Quoted prices in active markets (Level 1)**	**Significant other observable inputs (Level 2)**	**Significant unobservable inputs (Level 3)**
Asset:				
Marketable securities, available-for-sale	$2,957,376	$2,957,376	$—	$—
Liability:				
Derivative liability related to warrants	$22,248,040	$—	$—	$22,248,040

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The following table sets forth a summary of changes in the estimated fair value of the Company's Level 3 liability for the nine months ended September 30, 2013:

	Fair Value Measurements of Common Stock Warrants Using Significant Unobservable Inputs (Level 3)
Balance at January 1, 2013	$—
Issuance of common stock warrants:	
February 14, 2013	4,505,605
August 14, 2013	328,561
August 15, 2013	9,201,487
Total value upon issuance	14,035,653
Change in fair value of common stock warrant liability	8,198,672
Balance at September 30, 2013	$22,234,325

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 Topic 820. At each reporting period, all assets and liabilities for which the fair value measurement is based on significant unobservable inputs or instruments which trade infrequently and therefore have little or no price transparency are classified as Level 3.

NOTE 8. LICENSE AGREEMENT

On February 16, 2012 the Company entered into an agreement pursuant to which a biotech company (the "Sublicensor") with license rights to certain drug technologies agreed to grant us a worldwide sublicense for the development, manufacture and commercialization of a drug technology which is referred to as DARA, which is an Angiotesin Receptor Blocker ("ARB") and Endothelin Receptor Antagonist ("ERA") which the Company is initially using in connection with the treatment of focal segmental glomerulosclerosis ("FSGS") and which we refer to as RE-021. The sublicense agreement also enables the Company to sell the licensed technology as a research product or sublicense the technology to other third parties as potential sources of revenue. Under the license agreement, Sublicensor is obligated to transfer to the Company certain information, records, regulatory filings, materials and inventory controlled by Sublicensor and relating to or useful for developing RE-021. The Company must use commercially reasonable efforts to develop and commercialize RE-021 in specified major market countries and other countries in which the Company believes it is commercially reasonable to develop and commercialize such products. The agreement shall continue until neither party has any obligations under the agreement to make payments to the other party.

In accordance with the agreement as amended most recently as of January 7, 2013, the Company made two non-refundable payments totaling $2,550,000, the first payment of $1,150,000 made upon execution and the second payment of $1,400,000 was made in February 2013, which includes a $250,000 fee payable to the sublicensee in exchange for extended due date of this payment from October 1, 2012 to February 2013. As of September 30, 2013, the Company has recognized $2,300,000 for the cost of the License Agreement which is presented in the accompanying condensed consolidated balance sheet as an intangible asset that is being amortized on a straight-line basis over the term of the License Agreement which expires on September 30, 2023. The $250,000 of extension fees were expensed to operations in February 2013. In addition, the Company issued 620,000 common shares to Ligand valued at $1,550,000 as a result of the merger transaction, the amount of which was expensed to operations in December 2012. For the three and nine months ended September 30, 2013 and 2012, and for the period from

March 11, 2011 (inception) through September 30, 2013, the Company recognized amortization expense of the license related to this agreement of $51,065, $34,885, $151,531, $71,466, and $272,914 respectively.

The Company's sublicense agreement with Ligand specifically provides rights for the global development, manufacture, and commercial utilization of certain compounds, for any and all medical applications. The Company purchased this license in a bargained exchange transaction that was conducted at arm's length with an unrelated party. ASC 350 states that "Intangible assets that are acquired individually or with a group of assets in a transaction other than a business

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combination or an acquisition by a not-for-profit entity may meet asset recognition criteria in FASB Concepts Statement No. 5, Recognition and Measurement in Financial Statements of Business Enterprises."

The sublicense agreement provides broad and exclusive rights to use a technology based intangible that is a legally protected, separable, and has a stand-alone value independent of the Company or any other holder. The Company specifically considered the nature of the license, the rights to its use and whether the license itself meets the legal contractual and/or separability criterion described in under ASC 805 "Business Combinations."

The specific technology licensed under this agreement is known as DARA. This technology is fully developed, protected by issued patents and pending patent applications; the technology is the product of a long-term development effort that cannot be replicated. The DARA technology embodies unique know-how, including all biological, chemical, pharmacological toxicological, clinical, manufacturing assay and related data. This technology is also not generally known. Under ASC 805, intangible assets whose future economic benefits are legally protected are deemed to have met the legal contractual criteria and would therefore be accounted for as a separately identifiable intangible asset.

In addition to the above, the technology has a stand-alone value that makes it capable of being separated or divided from the Company or any other acquirer. The technology can be sold, transferred, licensed, rented, or exchanged either individually or together with a related contract, identifiable asset, or liability. The Company believes that the purchase of the license and the fact that the Company or any other market participant in possession of this license or a similar type of license would be able to sell, license, or otherwise exchange this technology for something else of value, provides evidence of its separability.

Further, the licensed technology could have use in other stand-alone broad applications outside of the initial intended use for FSGS, including hypertension and other nephrotic conditions. In connection with the acquired rights, the Company also acquired the right to enter into future sublicense agreements and has the right to transfer/sell this right to other third parties. Accordingly, the right to enter into sublicense agreements could be divided from other assets and independently sold. Consequently, this right meets the separability criterion specified in ASC 805-20-55-3 (e.g. it is an acquired intangible asset that is capable of being licensed).

NOTE 9. NOTES PAYABLE

Note Payable—related party

On February 1, 2012, the Company entered into a secured promissory note with a related party in the amount of $900,000, with an interest rate of 12% per annum, compounded monthly. The outstanding principal and interest balance of this note was fully repaid during the first quarter of 2013.

Total interest expense recognized for the three and nine months ended September 30, 2013 and 2012, and for the period from March 11, 2011 (inception) through September 30, 2013 amounted to $0, $26,761, $19,733, $70,559 and $147,480, respectively.

NOTE 10. RELATED PARTY TRANSACTIONS

On December 8, 2011, the Company received advances of funds aggregating $8,500 from entities related through common ownership. Such advances were repaid during the first quarter of 2013.

In August 2012, the Company paid a security deposit on behalf of an affiliate of $137,547 in connection with a building lease entered into by such affiliate. The Company assumed the lease from its affiliate in April 2013, whereby the security deposit was assigned to the Company. The Company leases approximately 4,216 square feet of office space for approximately $275,000 annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes, under a lease agreement expiring August 2016.

In the second quarter of 2013, the Company, its Chief Executive Officer and a related party, which is a former investor in the Company that was previously managed by the Company's Chief Executive Officer, became party to a series of agreements to settle up to $2,284,511 of liabilities, which Company management believes are the primary obligation of the related party. The Company and the related party have entered into indemnification agreements whereby the related party has agreed to defend and hold the Company harmless against all such obligations and amounts, whether paid or unpaid, arising from these agreements. Notwithstanding the indemnification, the Company recorded a $2,284,511 charge to operations during the quarter ended September 30, 2013 that was offset by a corresponding liability of $1,691,400 for the difference between (a) the aggregate amount of all such settlements, and (b) $593,111 of cash and non-cash

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consideration that the Company paid to immediately settle a portion of the agreement on behalf of the related party. The $1,691,400 is entirely paid as of the date of this filing. In addition, the Chief Executive Officer also agreed to provide one of the counter parties with 47,128 shares of his common stock in the Company as a separate component of one of these settlement agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the third quarter ended September 30, 2013. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

Concurrent with the execution of such settlement agreements, the Company received promissory notes from the related party whereby the related party agreed to pay the Company the principal amount of $593,111 plus interest at an annualized rate of 5% as reimbursement of the payments that the Company made to settle a portion of the agreements.

In October 2013, the Company paid $1,655,000 in cash and 5,000 shares of common stock valued at $36,400 to settle agreements on behalf of a related party. Upon payment, the Company and the related party entered into promissory notes whereby the related party agreed to pay the Company the principal amount of $1,691,400 plus interest at an annualized rate of 5% as reimbursement of payments that the Company made to settle a portion of the agreements.

The Company applied the accounting guidance provided in ASU 2013-04. The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount that the entity has a) contractually agreed to pay, and b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company has recorded the full amount of the settlements as a charge to its operations due to uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary. Any amounts that the Company may recover under the note due from the related party or under the terms of the indemnification agreement, if in fact any amounts are recovered at all, would be characterized as a capital contribution at the date such payments are received.

On August 15, 2013, the Company closed a private placement and sold 5,531,401 shares of the Company’s common stock, at a purchase price of $4.50 per share, or $24,891,303 in the aggregate, and warrants to purchase up to an aggregate of 2,765,701 shares of common stock with an exercise price of $6.00 per share underlying each warrant. Members of the Company’s management purchased an aggregate of 10,522 shares of common stock and warrants to purchase up to an aggregate of 5,261 shares of common stock in such private placement. The Warrants are deemed to be derivative instruments due to a ratchet provision that adjusts the exercise price if the Company issues additional equity instruments in the future at an effective price per share less than the exercise price then in effect. The issuance of the shares of common stock in such private placement was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

NOTE 11. STOCKHOLDERS’ DEFICIT

Issuances

Common Stock

In January 2013, the Company sold an aggregate of 272,221 shares of common stock, at a purchase price of $3.00 per share in certain private placement transactions, for an aggregate purchase price of $816,664 in cash. The issuance of such shares of common stock was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

On February 14, 2013, the Company closed a private placement (the “February Private Placement”) of 3,045,929 shares of common stock, at a purchase price of $3.00 per share, or $9,137,787 in the aggregate, and warrants (the “Warrants”) to purchase up to an aggregate of 1,597,969 shares of common stock with an exercise price of $3.60 per such share underlying any Warrant. The Warrants are deemed to be derivative instruments due to a ratchet provision that adjusts the exercise price if the Company issues additional equity instruments in the future at an effective price per share less than the exercise price then in effect. Upon issuance of the warrants, the Company recorded a liability of $4,505,605 to derivative financial instruments in its balance sheet. The issuance of such shares of common stock was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

On August 15, 2013, the Company closed a private placement and sold 5,531,401 shares of the Company's common stock, at a purchase price of $4.50 per share, or $24,891,303 in the aggregate, and warrants to purchase up to an aggregate of 2,765,701 shares of common stock with an exercise price of $6.00 per share underlying each warrant. The

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Warrants are deemed to be derivative instruments due to a ratchet provision that adjusts the exercise price if the Company issues additional equity instruments in the future at an effective price per share less than the exercise price then in effect. Upon issuance of the warrants, the Company recorded a liability of $9,201,487 to derivative financial instruments in its balance sheet. The issuance of the shares of common stock in such private placement was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

February Registration Rights Agreement

On February 14, 2013, in connection with the closing of the February Private Placement, the Company entered into a Registration Rights Agreement (the "Registration Rights Agreement") with the purchasers in the February Private Placement (the "Purchasers"), which sets forth the rights of the Purchasers to have their shares of common stock purchased in the February Private Placement and shares of common stock issuable upon exercise of the Warrants registered with the SEC for public resale.

Pursuant to the Registration Rights Agreement, the Company was required to file a Registration Statement on Form S-1 (the "Registration Statement") with the SEC within 30 days of the date of the Registration Rights Agreement registering the total number of shares of common stock purchased in the February Private Placement and shares of common stock issuable upon exercise of the Warrants. The Company further agreed to use its reasonable efforts to have the Registration Statement declared effective within 60 days after the date of the Registration Rights Agreement (or, in the event of a "full review" by the SEC, within 90 days after the date of the Registration Rights Agreement). The Company has also agreed to use reasonable efforts to maintain the effectiveness of the Registration Statement until all of the securities covered by the Registration Statement have or may be sold by investors under Rule 144 of the Securities Act, without volume or manner-of-sale restrictions.

The Registration Rights Agreement provided that in the event the Registration Statement was not filed or declared effective within the prescribed time period or if the Company failed to maintain the effectiveness of the Registration Statement as required for specified time periods, the Company shall pay to the holders of registrable securities, on the date of each such event and on each monthly anniversary thereof until the applicable event is cured, partial liquidated damages equal to 2.0% of the aggregate purchase price paid by such Purchaser in the February Private Placement, up to a maximum of 10.0% of such aggregate purchase price. If the Company fails to pay any partial liquidated damages pursuant to this Section in full within seven days after the date payable, the Company will pay interest thereon at a rate of 18% per annum (or such lesser maximum amount that is permitted to be paid by applicable law) to the Purchaser, accruing daily from the date such partial liquidated damages are due until such amounts, plus all such interest thereon, are paid in full.

The Company determined, as of the date of the financing transaction, that it was probable that it would not be in a position to cause the registration statement to be declared effective within the contractually defined time period. Accordingly, the Company allocated approximately $360,000 of the proceeds to a registration payment arrangement liability on the date that the financing transaction closed, in accordance with the guidelines of ASC 825-20. As described in Note 3, the Company and the investors who are parties to the registration payment arrangement entered into an the Amended Registration Rights Agreement which provides, among other things, for a waiver of the liquidated damages that the Company incurred under the original terms of the registration payment arrangement described herein. The Company recognized $360,000 as income upon the waiver of the liquidated damages.

First Amendment to the February Registration Rights Agreement

As described in Note 3, the Company and the investors who participated in the private placement transaction that the Company completed on February 14, 2013 entered into the Amended Registration Rights Agreement which provides, among other things, for (i) a waiver of any and all liquidated damages that the Company incurred for its inability to cause a registration statement to be declared effective within certain contractually defined time-frames stipulated in the original agreement; (ii) a commitment on the part of the investors in the February private placement to participate in the private placement transaction that the Company completed on August 15, 2013; and (iii) a covenant on the part of the Company to proceed with the sale of shares that were issued in the August 15, 2013 private placement transaction. In exchange, the Company paid an aggregate fee of $2,495,256 to these investors consisting of (i) 73,710 shares of the Company's common stock with an aggregate fair value of $331,695 (based on the selling price of $4.50 per share in the August financing transaction); (ii) cash in the amount of $1,835,000; and (iii) warrants to purchase

98,756 shares of common stock with a fair value of $328,561 that were classified as derivative liability instruments. The investors were also given the option to purchase shares of the Company's common stock at $4.50 per share as a use of the cash portion of the payment arrangement. Accordingly, $946,196 of the cash portion of the fee was settled in cash and the remainder was settled by

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the issuance of 197,512 shares. Additionally, the Company paid $103,425 to an investor to whom the Company sold shares in a private placement transaction in January 2013 and who participated in the August 2013 private placement transaction. This payment was settled entirely by the issuance of 20,685 shares of the Company's common stock at a value of $5.00 per share.

The Company recorded the aggregate amount of the payments made to the investors by to allocating approximately $360,000 to the waiver of the original registration payment obligation taken as a charge to operations and the remaining amount of $2,238,681 is treated as reduction of the proceeds received in the August financing transaction.

August Registration Rights Agreement

On August 15, 2013, in connection with the closing of the August 15, 2013 private placement (the "August Private Placement"), the Company entered into a Registration Rights Agreement (the "Registration Rights Agreement") with the purchasers in the August Private Placement (the "Purchasers"), which sets forth the rights of the Purchasers to have their shares of common stock purchased in the Private Placement and shares of common stock issuable upon exercise of the Warrants registered with the SEC for public resale.

Pursuant to the Registration Rights Agreement, the Company was required to file a Registration Statement on Form S-1 (the "Registration Statement") with the SEC within 30 days of the date of the Registration Rights Agreement registering the total number of shares of common stock purchased in the August Private Placement and shares of common stock issuable upon exercise of the Warrants. The Company further agreed to use its reasonable efforts to have the Registration Statement declared effective within 60 days after the date of the Registration Rights Agreement (or, in the event of a "full review" by the SEC, within 120 days after the date of the Registration Rights Agreement). The Company has also agreed to use reasonable efforts to maintain the effectiveness of the Registration Statement until all of the securities covered by the Registration Statement have or may be sold by investors under Rule 144 of the Securities Act, without volume or manner-of-sale restrictions.

The Registration Rights Agreement provided that in the event the Registration Statement was not filed or declared effective within the prescribed time period or if the Company failed to maintain the effectiveness of the Registration Statement as required for specified time periods, the Company shall pay to the holders of registrable securities, on the date of each such event and on each monthly anniversary thereof until the applicable event is cured, partial liquidated damages equal to 2.0% of the aggregate purchase price paid by such Purchaser in the August Private Placement, up to a maximum of 10.0% of such aggregate purchase price. If the Company fails to pay any partial liquidated damages pursuant to this Section in full within seven days after the date payable, the Company will pay interest thereon at a rate of 18% per annum (or such lesser maximum amount that is permitted to be paid by applicable law) to the Purchaser, accruing daily from the date such partial liquidated damages are due until such amounts, plus all such interest thereon, are paid in full.

On September 13, 2013, the Company submitted the Registration Statement to the SEC on a confidential basis. The Company determined, as of the date of the financing transaction, that it was probable that it would be in a position to cause the registration statement to be declared effective within the contractually defined time period.

Stock Options

On May 13, 2013, the Company issued options (the "Options") to purchase 120,000 shares of common stock in connection with an employment agreement with Horacio Plotkin, M.D. (the "Plotkin Employment Agreement") pursuant to which Dr. Plotkin was appointed as Chief Medical Officer of the Company. The options vest quarterly in pro rata portions during the 3 years following the effective date of July 1, 2013. The Company valued these Options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest rate of .83% (based on the US Treasury note yield), expected term (in years) of 5.81 (based on guidance provided in SAB 107 that allows the Company to use the simplified method for "plain vanilla" options for this calculation), expected volatility of 98.56% (based on historical stock volatilities of several comparable publicly-traded companies over a period equal to the expected term of the options, as the Company does not have a long trading history to estimate the volatility of its own common stock), and an exercise price equal to the fair value of the stock on the date of issuance of $8.70 per share. For the three and nine months ended September 30, 2013 the Company recognized $64,716 and $101,399 as compensation expense related to the Options. At September 30, 2013, the unrecognized compensation expense, remaining amortization period, intrinsic value and remaining contract life of the Options are $703,333, 2.5 years, $0 and 9.62 years, respectively.

On September 9, 2013, the Company issued options to purchase 90,000 shares of common stock to two employees. The options vest quarterly in pro rata portions during the 3 years following the effective date of July 1, 2013. The Company valued these options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest

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rate of 1.71% (based on the US Treasury note yield), expected term (in years) of 5.81 (based on guidance provided in SAB 107 that allows the Company to use the simplified method for "plain vanilla" options for this calculation), expected volatility of 104.78% (based on historical stock volatilities of several comparable publicly-traded companies over a period equal to the expected term of the options, as the Company does not have a long trading history to estimate the volatility of its own common stock), and an exercise price equal to the fair value of the stock on the date of issuance of $6.20 per share. For the three and nine months ended September 30, 2013 the Company recognized $9,606 as compensation expense related to the options. At September 30, 2013, the unrecognized compensation expense, remaining amortization period, intrinsic value and remaining contract life of the options are $438,748, 2.5 years, $0 and 9.94 years, respectively.

On August 13, 2013, the Company issued options to purchase 50,000 shares of common stock to an employee. The options vest quarterly in pro rata portions during the 3 years beginning on December 31, 2013.

NOTE 12. INCENTIVE SHARES

At September 30, 2013, the Company did not have any active share-based compensation plans available for grants to employees, non-employee directors and consultants. Since its inception, the Company has granted incentive shares. For the three and nine months ended September 30, 2013 and 2012, and for the period from March 11, 2011 (inception) through September 30, 2013, the Company recognized $1,741,908, $7,027,675, $2,029,505, $14,014,402 and $26,419,022 as compensation expense related to incentive shares granted in the consolidated statements of operations, respectively. Share compensation for non-employee awards subject to vesting is being accrued at current fair value. As of September 30, 2013, there was approximately $1,294,232, of unrecognized compensation cost related to incentive shares issued. This amount is expected to be recognized over a weighted average of 2.25 years.

	Employee — number of shares	Non Employee — number of shares	Total number of shares	Weighted Average Fair Value
Unvested December 31, 2011	1,281,225	321,165	1,602,390	$4.00
Granted	866,180	87,503	953,683	12.89
Vested	(2,048,280)	(193,672)	(2,241,952)	7.34
Forfeited	(46,353)	—	(46,353)	9.06
Unvested December 31, 2012	52,772	214,996	267,768	5.79
Granted	135,000	—	135,000	6.24
Vested	(17,918)	(115,445)	(133,363)	5.05
Forfeited	(20,833)	(37,500)	(58,333)	4.00
Unvested September 30, 2013	149,021	62,051	211,073	$6.43

All of the Company's share base payments were originally issued as Retrophin LLC Class B incentive units that represent a profits interest up through the date of Retrophin LLC's conversation to a C Corporation, which was structured as a tax free exchange transaction.

Shares granted as incentive shares were originally subject to certain conditions at the time of grant. Such conditions specified that upon the occurrence of a Termination Event, as defined in the amended operating agreement the Company shall have the right, but not the obligation, to repurchase, all, of the vested incentive shares owned by such incentive shareholder, at a purchase price based on the fair market value of the incentive shares determined in good faith by the Board of Directors. The aforementioned repurchase option was rescinded upon the Company's conversion to a corporation.

Effective May 20, 2013, the Company entered into an employment agreement with Marc L. Panoff (the "Panoff Employment Agreement") pursuant to which Mr. Panoff was appointed as Chief Financial Officer and Chief Accounting Officer of the Company. In accordance with the terms of the Panoff Employment Agreement, Mr. Panoff will be granted 120,000 units of restricted common stock of the Company, a pro rata portion of which will vest quarterly beginning on December 31, 2013 during the 3 years following the effective date.

On July 1, 2013, the Company granted 15,000 units of restricted common stock of the Company to an employee. The stock will vest quarterly in pro rata portions beginning September 30, 2013 during the 3 years following the grant date.

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NOTE 13. COMMITMENTS AND CONTINGENCIES

Leases

The Company assumed a building lease from an affiliate in April 2013 for office space at its principal offices in New York, New York and is responsible for rent of approximately $275,000 annually plus rent escalations through August 2016 (see Note 10).

On October 1, 2013, the Company entered into building lease for office space in Cambridge, Massachusetts and is responsible for rent of approximately $216,000 annually plus rent escalations through September 2016 (see Note 14).

On October 8, 2013, the Company entered into an amended lease agreement for additional office space at its principal offices in New York, New York and is responsible for additional rent of approximately $225,000 annually plus rent escalations through August 2016 (see Note 14).

Exclusivity Agreements

On August 16, 2013, the Company announced that it had signed an agreement with a major pharmaceutical company for the exclusive right to negotiate a royalty-bearing U.S. license for a product to be developed for the treatment of Autism and Schizophrenia. Pursuant to the exclusivity agreement, the Company paid the major pharmaceutical company a non-refundable upfront fee of $2 million and will have an exclusive period of 120 days to negotiate a license agreement (see Note 3). The Company is in active negotiations to consummate a license agreement. If a definitive license agreement is consummated, the Company will apply the upfront fee to the license and will make a determination as to whether it should be treated as an asset or research and development expense. If no definitive agreement is consummated, the upfront fee will be expensed.

On September 20, 2013, the Company signed an agreement with an individual for the exclusive right to negotiate a royalty-bearing U.S. license for a product to be developed for the treatment of central nervous system disorders. Pursuant to the exclusivity agreement, the Company paid the individual a non-refundable upfront fee of $250,000 and will have an exclusive right until December 31, 2013. If an agreement is not executed on or before December 31, 2013, the Company will receive a partial refund of the upfront fee. Upon execution of a license agreement, the Company would receive the exclusive right to the intellectual property to develop, manufacture and sell the product worldwide and would pay additional fees to the major pharmaceutical company. The Company is in active negotiations to consummate a license agreement. If a definitive license agreement is consummated, the Company will apply the upfront fee to the license and will make a determination as to whether it should be treated as an asset or research and development expense. If no definitive agreement is consummated, the upfront fee will be expensed.

For the three and nine months ended September 30, 2013, the Company has recorded $2,250,000 as exclusivity agreement deposits to secure license exclusivity under current assets (see Note 3).

Research Agreement

Effective October 1, 2013, the Company signed a Sponsored Research Agreement (“SRA”) with St. Jude (see Note 14).

NOTE 14. SUBSEQUENT EVENTS

On October 1, 2013 the Company entered into a building lease for approximately 4,232 square feet of office space located in Cambridge, MA. The Company is responsible for approximately $216,000 annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes, under a lease agreement expiring in September 2016.

On October 8, 2013, the Company amended its lease agreement for its principal office located in New York City. The Company expanded its principal office and is responsible for additional rent of approximately $225,000 annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes, under a lease agreement expiring in September 2016.

Research Agreement

Effective October 1, 2013, the Company signed a SRA with St. Jude. The Company is responsible for a total of $780,674 payable in four equal installments on October 19, 2013, March 19, 2014, September 19, 2014, and March 19, 2015. Unless otherwise terminated by operation of law or by acts of the parties in accordance with the terms of the agreement, the SRA shall be in full force and effect for a period of two (2) years and shall expire on October 1, 2015. The term may be extended by written agreement between the parties.

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Settlement Payments

In October 2013, the Company paid $1,655,000 in cash and 5,000 shares of common stock valued at $36,400 to settle agreements on behalf of a related party. Upon payment, the Company and the related party entered into promissory notes whereby the related party agreed to pay the Company the principal amount of $1,691,400 plus interest at an annualized rate of 5% as reimbursement of payments that the Company made to settle a portion of the agreements. The Company applied the accounting guidance provided in ASU 2013-04. The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount that the entity has a) contractually agreed to pay, and b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company has recorded the full amount of the settlements as a charge to its operations due to uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary. Any amounts that the Company may recover under the note due from the related party or under the terms of the indemnification agreement, if in fact any amounts are recovered at all, would be characterized as a capital contribution at the date such payments are received.

Additional Events Subsequent to November 13, 2013 (unaudited)

On December 1, 2013, the Company entered into a lease for approximately 2,500 square feet of office space located in Carlsbad, CA that expires in February, 2017. The Company is responsible for approximately $70,500 of annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes.

Director Compensation

On December 6, 2013, the Company's board of directors established a compensation policy for the Company's non-employee directors pursuant to which each non-employee director shall receive $100,000 annually, which amount shall be comprised of not more than $25,000 in cash, with the remainder paid in the form of options to purchase shares of the Company's common stock. Each non-employee director may, at his discretion, determine to receive less than $25,000 annually in the form of cash, in which case such amount will be paid to such director in the form of options to purchase additional shares of the Company's common stock. In accordance with such policy, in December 2013, the Company issued options to purchase 51,000 shares of common stock to four non-employee directors. Such options vest immediately and are exercisable over a ten year period at an exercise price of $8.70 per share.

Stock Options

On December 6, 2013, the Company's board of directors approved the grant of options to purchase an aggregate of 330,000 shares of the Company's common stock to six employees. Such options vest quarterly over the three year period from the grant date and are exercisable over a ten year period at an exercise price of $8.70 per share.

Novartis License

On December 12, 2013, the Company entered into an agreement with Novartis and Novartis AG pursuant to which Novartis and Novartis AG agreed to grant the Company an exclusive, perpetual, and royalty-bearing license for the manufacture, development and commercialization of Syntocinon and related intranasal products in the United States. Under the license, Novartis and Novartis AG are obligated to transfer to the Company certain information that is necessary for or related to the development or commercialization of Syntocinon. The Company is responsible for conducting research and preclinical, clinical and other development of Syntocinon at its expense, and must use commercially reasonably efforts to develop Syntocinon in the United States.

As consideration for the license, the Company paid to Novartis and Novartis AG a $5 million upfront fee and is required to make substantial payments upon the achievement of certain milestones. Should the Company commercialize Syntocinon, it will be obligated to pay Novartis and Novartis AG a 20% royalty on net sales of such products. The Company also required to pay annual maintenance fees to Novartis and Novartis AG.

The license agreement contains other customary clauses and terms as are common in similar agreements in the industry. The license agreement will continue in perpetuity unless terminated by the Company or by Novartis and Novartis AG.

The Company is currently evaluating the accounting treatment to determine whether the upfront and future payments should be treated as a capitalized asset or as research and development expense.

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Central Nervous System License

On December 12, 2013, the Company entered into an agreement "Weg License Agreement," with Stuart Weg, MD, pursuant to which Dr. Weg agreed to grant the Company an exclusive worldwide license for the manufacture, development and distribution of products to be developed for the treatment of central nervous system disorders. As consideration for the license, the Company is required to pay Dr. Weg an upfront fee, which amount included a $250,000 payment prior to the execution of the Weg License Agreement, as well as certain maintenance and sublicensing fees. The Company is also obligated to pay Dr. Weg certain royalties on sales of FDA-approved products.

The Weg License Agreement contains other customary clauses and terms as are common in similar agreements in the industry.

The Weg License Agreement will continue in perpetuity unless terminated by the Company or by Dr. Weg. The Company may terminate the agreement at any time by giving written notice to Dr. Weg. Dr. Weg may terminate the agreement due to the Company's uncured material breach of the agreement.

The Company is currently evaluating the accounting treatment to determine whether the upfront and future payments should be treated as a capitalized asset or as research and development expense.

UCSD Sponsored Research Agreement

On December 12, 2013, the Company entered into an agreement with The Regents of the University of California, on behalf of its San Diego Campus ("UCSD"), pursuant to which UCSD will undertake research projects related to a study on oxytocin. As consideration for the research program, the Company is obligated to pay an aggregate of approximately $1.6 million in fees to UCSD on a specified timeline, of which $0 has been paid as of the date hereof. This agreement will continue until completion of the projects, unless earlier terminated by either party (i) due to a material uncured breach of such agreement by the other party or (ii) for any reason by giving written notice to the other party.

Withdrawal of Transcept Proposal

On December 16, 2013, the Company announced that it had withdrawn its proposal to acquire all of the issued and outstanding shares of common stock of Transcept Pharmaceuticals, Inc. ("Transcept"). The Company no longer owns any shares of Transcept's common stock.

Shkreli Employment Agreement

On December 16, 2013, the Company entered into an employment agreement (the "Shkreli Employment Agreement") with Martin Shkreli, pursuant to which Mr. Shkreli will continue to serve as the Company's Chief Executive Officer. In accordance with the terms of the Shkreli Employment Agreement, Mr. Shkreli will be paid (i) a base salary in the amount of $300,000 (subject to adjustments at the discretion of the Company's board of directors after each anniversary of the Effective Date), and (ii) at the sole discretion of the board, an annual bonus award based upon specific goals and performance metrics. Mr. Shkreli will also be awarded options to purchase One Million Eighty Thousand (1,080,000) shares of restricted common stock of the Company, a pro rata portion of which will vest quarterly during the 3 years following the Effective Date. In the event of a change of control of the Company, all of Mr. Shkreli's unvested options shall immediately vest.

The Shkreli Employment Agreement contemplates that Mr. Shkreli's employment will be for a three-year term and may be automatically extended for successive three-year periods unless (i) Mr. Shkreli gives notice of non-extension to the Company no later than one hundred eighty (180) days prior to the expiration of the Agreement or (ii) Mr. Shkreli is terminated.

In the event Mr. Shkreli's employment is terminated by Mr. Shkreli for good reason (as such term is defined in the Shkreli Employment Agreement), then Mr. Shkreli will be entitled to continue to receive his annual base salary, any unpaid bonus and health insurance coverage on the same terms as made available to the Company's employees for a period of twelve (12) months following such termination. If Mr. Shkreli's employment is terminated other than for good reason, Mr. Shrekli will forfeit any unvested stock options that he received and will not be entitled to severance or any additional payments.

If Mr. Shkreli's employment is terminated for cause (as such term is defined in the Shkreli employment Agreement) then Mr. Shkreli will not be entitled to any further payments of any kind, except for payment of base salary plus reimbursement of certain expenses.

In the event that Mr. Shkreli is no longer employed by the Company, any options that have not vested prior to the date of termination will be immediately cancelled and not subject to further vesting.

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Stock Repurchases

In the fourth quarter of 2013 and early in the first quarter of 2014, the Company repurchased approximately 264,000 shares of its common stock for an aggregate purchase price of approximately $1.9 million. The Company currently recognizes such repurchased common stock as treasury stock.

Acquisition of Kyalin Biosciences

On December 23, 2013, the Company entered into, and consummated the transactions contemplated by, a stock purchase agreement (the "Stock Purchase Agreement") with Kyalin Biosciences, Inc., a Delaware corporation ("Kyalin") and the sellers signatory thereto (the "Sellers"), pursuant to which the Company acquired all of the issued and outstanding shares of capital stock (the "Shares"), of Kyalin. In consideration for the Shares, the Company agreed to pay to the Sellers (i) $1 million of cash consideration at specified dates; and (ii) up to $4 million of the Company's common stock, par value $0.0001 per share ("Common Stock") at certain dates and subject to the achievement of certain milestones. Under certain limited circumstances, the Company would be required to pay to the Sellers, in the place of such shares of Common Stock, an amount of cash equal to one-half (1/2) of the value of the shares of Common Stock issuable in accordance with the Stock Purchase Agreement. In connection with such acquisition, the Company hired Srinivas Rao, M.D., Ph.D., the Founder and President of Kyalin.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of

Retrophin, Inc. and Subsidiary

We have audited the accompanying consolidated balance sheets of Retrophin, Inc. and Subsidiary (a development stage company) (the "Company") as of December 31, 2012 and 2011 and the related consolidated statements of operations, changes in stockholder's deficit and cash flows for the year ended December 31, 2012, for the periods from March 11, 2011 (inception) through December 31, 2011 and March 11, 2011 (inception) through December 31, 2012. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

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

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the consolidated financial position of Retrophin, Inc. and Subsidiary (a development stage company) as of December 31, 2012 and 2011, and the consolidated results of its operations and its cash flows for the year ended December 31, 2012, for the periods from March 11, 2011 (inception) through December 31, 2011 and from March 11, 2011 (inception) through December 31, 2012, in conformity with accounting principles generally accepted in the United States of America.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 2 to the consolidated financial statements, the Company is a development stage enterprise with no revenues, historical losses and limited capital resources. The Company, as a development stage enterprise, is subject to risks and uncertainties as to whether it will be able to raise capital and commence its planned operations. These conditions raise substantial doubt about the Company's ability to continue as a going concern. Management's plans regarding these matters also are described in Note 2. The consolidated financial statements do not include any adjustments relating to the recovery of assets or classification of liabilities might be necessary should the Company be unable to continue as a going concern.

/s/ Marcum LLP

New York, NY

June 13, 2013, except paragraph numbers 3, 4, 5 and 6 of Note 2 and paragraph numbers 3, 8, 9 and 10 of Note 12, as to which the date is September 13, 2013.

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONSOLIDATED BALANCE SHEETS

	December 31, 2012	December 31, 2011
Assets		
Current assets		
Cash	$11,388	$10,053
Other current assets	21,830	7,000
Total current assets	33,218	17,053
Property and equipment, net	23,790	2,517
Patents pending	18,093	—
Due from affiliate	137,547	
Technology license, net	2,178,617	—
Total assets	$2,391,265	$19,570
Liabilities and Stockholders' Deficit		
Liabilities		
Current liabilities		
Technology license liability	$1,300,000	$—
Accounts payable	1,023,320	340,134
Accrued expenses	2,467,796	169,721
Note payable—related party	884,764	—
Investors deposit	100,000	—
Due to related parties	23,200	46,000
Total liabilities	5,799,080	555,855
STOCKHOLDERS' DEFICIT		
Preferred stock Series A $0.001 par value; 20,000,000 authorized; 0 and 0 issued and outstanding, respectively	—	—
Common stock $0.0001 par value; 100,000,000 authorized; 8,952,905 and 4,042,265 issued and outstanding, respectively	895	404
Additional paid-in capital	30,203,402	2,766,567
Subscription receivable—Stockholder	—	(35,000)
Deficit accumulated during the development stage	(33,612,112)	(3,268,256)
Total stockholders' deficit	(3,407,815)	(536,285)
Total liabilities and stockholders' deficit	$2,391,265	$19,570

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONSOLIDATED STATEMENTS OF OPERATIONS

	For the year ended December 31, 2012	For the period from March 11, 2011 (inception) through December 31, 2011	For the period from March 11, 2011 (inception) through December 31, 2012
Operating expenses:			
Compensation and related costs—inclusive of share based compensation $16,012,850, $1,724,967 and $17,737,817	$18,133,550	$2,227,203	$20,360,753
Professional fees—inclusive of share based compensation $6,397,372, $254,332, and $6,651,704	9,035,702	909,681	9,945,383
Selling, general and administrative	1,292,296	63,812	1,356,108
Technology license contingent fees	1,700,000	—	1,700,000
Rent	95,469	63,000	158,469
Total operating expenses	30,257,017	3,263,696	33,520,713
Other income (expense):			
Interest income	21,830	75	21,905
Interest expense	(105,917)	—	(105,917)
Loss on transactions denominated in foreign currencies	(2,752)	(4,635)	(7,387)
Total other expense	(86,839)	(4,560)	(91,399)
Net loss	$(30,343,856)	$(3,268,256)	$(33,612,112)
Net loss per common share—basic and diluted	$(8.29)	$(1.59)	
Weighted average number of common shares outstanding during the period—basic and diluted	3,662,114	2,053,402	

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' DEFICIT

	Common stock		Additional paid in capital	Receivable due from stockholder	Accumulated deficit	Total Stockholders' deficit
	Shares	Amount				
Balance—March 11, 2011 (inception)	—	$—	$—	$—	$—	$—
Issuance of common shares	1,608,300	161	24,839	(25,000)	—	—
Issuance of common shares to founders in connection with the initial capital contribution	50,000	5	95	—	—	100
Incentive shares granted—employees	1,758,300	176	(176)	—	—	—
Incentive shares granted—non employees	381,000	38	(38)	—	—	—
Incentive shares forfeited—employees	(45,835)	(5)	5	—	—	—
Share based compensation—employees	—	—	1,724,967	—	—	1,724,967
Share based compensation—non employees	—	—	254,332	—	—	254,332
Issuance of shares in connection with March 2011 private placement, net of fees of $66,061	253,750	25	658,914	—	—	658,939
Issuance of Series A preferred in connection with March 2011 private placement, net of fees of $1,367, recapitalization to common stock	36,750	4	103,629	—	—	103,633
Loan made to stockholder	—	—	—	(10,000)	—	(10,000)
Net loss	—	—	—	—	(3,268,256)	(3,268,256)
Balance—December 31, 2011	4,042,265	404	2,766,567	(35,000)	(3,268,256)	(536,285)
Issuance of Series A preferred in connection with January 2012 private placement, net of fees of $61,677 recapitalized into common stock	326,963	33	1,806,644	—	—	1,806,677

	Common stock		Additional paid in capital	Receivable due from stockholder	Accumulated deficit	Total Stockholders' deficit
Issuance of Series A preferred in connection with May 2012 private placement, net of fees of $12,275, recapitalized into common stock	470,764	47	1,668,979	—	—	1,669,026
Shares transferred to consultants by founder for services rendered to the Company	—	—	4,400,000	—	—	4,400,000
Shares transferred to employees by founders for services rendered to the Company	—	—	1,375,000	—	—	1,375,000
Shares issued in accordance with technology license agreement	620,000	62	1,549,938	—	—	1,550,000
Shares outstanding at time of reverse merger completed on December 12, 2012	2,585,583	259	1,142	—	—	1,401
Incentive shares granted—employees	866,180	86	(86)	—	—	—
Incentive shares granted—non employees	87,503	9	(9)	—	—	—
Incentive shares forfeited—employees	(46,353)	(5)	5	—	—	—
Share based compensation—employees	—	—	14,637,850	—	—	14,637,850
Share based compensation—non employees	—	—	1,997,372	—	—	1,997,372
Receivable due from stockholder charged to compensation	—	—	—	407,900	—	407,900
Loan made to stockholder	—	—	—	(372,900)		(372,900)
Net loss	—	—	—	—	(30,343,856)	(30,343,856)
Balance—December 31, 2012	8,952,905	$895	$30,203,402	$—	$(33,612,112)	$(3,407,815)

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONSOLIDATED STATEMENTS OF CASH FLOWS

	For the year ended December 31, 2012	For the period from March 11, 2011 (inception) through December 31, 2011	For the period from March 11, 2011 (inception) through December 31, 2012
Cash Flows From Operating Activities:			
Net loss	$(30,343,856)	$(3,268,256)	$(33,612,112)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	124,885	355	125,240
Compensation in lieu of stockholder receivable	407,900	—	407,900
Share based compensation—employees	16,012,850	1,724,967	17,737,817
Share based compensation—non-employees	6,397,372	254,332	6,651,704
Share based payment—Technology license contingent fee	1,550,000	—	1,550,000
Changes in operating assets and liabilities:			
Other assets	(14,830)	(7,000)	(21,830)
Technology license fee	150,000	—	150,000
Accounts payable	680,865	340,134	1,020,999
Accrued expenses	2,298,075	169,721	2,467,796
Net cash (used) in operating activities	(2,736,739)	(785,747)	(3,522,486)
Cash Flows From Investing Activities:			
Purchase of fixed assets	(24,774)	(2,872)	(27,646)
Purchase of intangible assets	(1,168,093)	—	(1,168,093)
Cash received in merger transaction	3,721	—	3,721
Payments made on behalf of affiliate	(137,547)	—	(137,547)
Loans made to stockholder	(372,900)	(10,000)	(382,900)
Net cash (used) in investing activities	(1,699,593)	(12,872)	(1,712,465)
Cash Flows From Financing Activities:			
Proceeds from advances from related parties	10,500	46,000	56,500
Repayment of advances from related parties	(33,300)	—	(33,300)
Proceeds from note payable—related party	930,000	—	930,000
Repayment of note payable—related party	(45,236)	—	(45,236)
Investor deposit	100,000	—	100,000
Proceeds received from issuance of common stock, net of cost of $73,952, $67,428 and $141,380, respectively	3,475,703	762,672	4,238,375
Net cash provided in financing activities	4,437,667	808,672	5,246,339
Net decrease in cash	1,335	10,053	11,388
Cash, beginning of period	10,053	—	—
Cash, end of period	$11,388	$10,053	$11,388
Supplemental Disclosure of Cash Flow Information:			
Cash paid for interest	$14,764	$—	$14,764
Non-cash investing and financing activities:			

	For the year ended December 31, 2012	For the period from March 11, 2011 (inception) through December 31, 2011	For the period from March 11, 2011 (inception) through December 31, 2012
Issuance of common stock for subscription receivable	$—	$25,000	$25,000

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

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
NOTE 1.

- DESCRIPTION OF BUSINESS

Organization and Description of Business
Retrophin, Inc. (the "Company") was incorporated as Desert Gateway, Inc. ("Desert Gateway") in the State of Oklahoma on February 8, 2008. Desert Gateway was originally a wholly-owned subsidiary of American Merchant Data Services, Inc. ("American Merchant"). In a 2008 reorganization of American Merchant, each share of outstanding common stock of American Merchant was converted into one share of Desert Gateway, while all of American Merchant's operating assets, liabilities and tax attributes (including accumulated losses and net operating losses) carried forward to another subsidiary of American Merchant in a downstream merger with such other subsidiary. Accordingly, American Merchant is not considered a predecessor company of the Desert Gateway for accounting or legal purposes. Following the 2008 reorganization, Desert Gateway re-domiciled to Delaware. Since inception and until Desert Gateway's merger with Retrophin, Inc., a private company ("Former Retrophin") in December 2012 (as described below), Desert Gateway had no existing operations, and its sole purpose was to locate and consummate a merger or acquisition with a private entity.
Former Retrophin, Inc. was originally organized as a Delaware limited liability company, named Retrophin, LLC, on March 11, 2011 ("Inception"). On September 20, 2012, Retrophin filed a Certificate of Conversion to change its legal form of organization from a limited liability company to a corporation in the State of Delaware. This conversion (as more fully described in Note 8) into a corporation, which preceded the Merger on December 12, 2012, resulted in no change of ownership and was therefore considered a recapitalization of the LLC's equity.
On September 13, 2012, Former Retrophin formed a new entity, Retrophin Pharmaceutical, Inc., a Delaware corporation and a wholly-owned subsidiary of Retrophin, Inc.
On December 12, 2012, Desert Gateway completed the transactions contemplated under the Agreement and Plan of Merger, dated as of December 12, 2012 (the "Merger Agreement"), by and among Desert Gateway, Desert Gateway Acquisition Corp., a Delaware corporation and wholly-owned subsidiary of Desert Gateway, and Former Retrophin, our predecessor, in which Former Retrophin became a wholly-owned subsidiary and the principal operating subsidiary of the Company. The transactions contemplated by the Merger Agreement are collectively referred to herein as the "2012 Merger". The Merger became effective on December 12, 2012, upon the filing of a certificate of merger with the Secretary of State of the State of Delaware. Accordingly, the Merger resulted in a change in control of Desert Gateway. Desert Gateway's net assets amounted to $1,401 at the time of the merger, including $3,721 of cash and $2,320 of trade liabilities. The merger is being accounted for as a reverse merger and recapitalization of Former Retrophin into Desert Gateway, whereby Desert Gateway is the legal acquirer and Former Retrophin is the legal acquiree and the accounting acquirer in this transaction.
Upon the consummation of the Merger all of the issued and outstanding Class A Preferred shares of Former Retrophin were exchanged into the Company's common shares at the rate of 1 to 7 (each Class A Preferred stockholder received 7 shares of the Company's common stock) and all of the issued and outstanding share of common stock of Former Retrophin were exchanged for shares of the Company's common stock on exchange ratio of 1 to 5 (each Common stockholder of Former Retrophin received 5 shares of the Company's common stock).
The consolidated financial statements give retroactive effect to these changes as if the merger occurred at the inception of the Company.
On February 14, 2013, the Company changed its name to "Retrophin, Inc." through a short-form merger pursuant to Section 253 of the Delaware General Corporation Law, with its then wholly owned subsidiary, and our predecessor, Retrophin, with the Company continuing as the surviving corporation following the merger.
On April 1, 2013, the Company changed its fiscal year end from the last day of February to a fiscal year end of December 31 in order to confirm its reporting cycle to that of Former Retrophin.
Retrophin, is an emerging biotechnology company dedicated to developing drugs for rare and life-threatening diseases. Retrophin's primary business objective is to develop and commercialize therapies for orphan diseases, such

as Duchenne muscular dystrophy, or DMD. The Company is considered to be a development stage company and, as such, the Company's financial statements are prepared in accordance with the Accounting Standards Codification ("ASC") 915 "Development Stage Entities." The Company is subject to all of the risks and uncertainties associated with development stage companies.

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)

NOTE 2.

- LIQUIDITY AND FINANCIAL CONDITION AND MANAGEMENT'S PLANS

The Company incurred a net loss of approximately $33.6 million, including stock-based compensation charge of $24,389,521 for the period from March 11, 2011 (inception) to December 31, 2012. At December 31, 2012, the Company had a cash balance of approximately $11,000 and a working capital deficiency of approximately $5,766,000. The Company's accumulated deficit amounted to approximately $33,600,000 at December 31, 2012. The Company has principally financed its operations from inception using proceeds from sales of its equity securities in a series of private placement transactions (see Note 7). The Company to date has no revenues, significantly limited capital resources and is subject to all of the risks and uncertainties that are typical of a development stage enterprise. Significant uncertainties include, among others, whether it will be able to raise the capital it needs to finance the start of its planned operations and whether such operations, if launched, will enable the Company to become a profitable enterprise.

On February 14, 2013, in connection with the closing of a private placement, we issued and sold an aggregate of 3,045,929 shares of common stock, for an aggregate purchase price of $9,137,787 in cash, and warrants to purchase up to an aggregate of 1,522,969 shares of common stock. The Company concurrently entered into a registration payment arrangement requiring it to file a Form S-1 with the Securities and Exchange Commission within 30 days of the closing date of this transaction and cause it to be declared effective by no later than 90 days of the closing date of this transaction. The registration payment arrangement provided for the Company to pay liquidated damages in the amount of 2% of the proceeds received in this transaction per month for each month that the Company is not in compliance with this requirement, not to exceed 10% in the aggregate. The Company determined that it was probable that it would not be in a position to comply with these requirements and therefore allocated approximately $360,000 of the proceeds received in this transaction to a registration payment obligation.

In the second quarter of 2013, the Company, its Chief Executive Officer and a related party became party to a series of agreements to settle up to $2,284,511 of liabilities, which Company management believes are the primary obligation of the related party. The Company and the related party have entered into indemnification agreements whereby the related party has agreed to defend and hold the Company harmless against all such obligations and amounts, whether paid or unpaid, arising from these agreements. The Company paid $593,111 of the total settlement liabilities in the second quarter of 2013 and had $1,691,400 in outstanding settlement liabilities, $300,000 of which is past due, $713,900 of which was due in July 2013, and $677,500 of which was due in August 2013. The counter parties to these agreements reserve the right to demand payment at any time. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock in the Company to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the second quarter ended June 30, 2013.

There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

Concurrent with the execution of such settlement agreements, the Company received promissory notes from the related party whereby the related party agreed to pay the Company the principal amount of $593,111 plus interest at an annualized rate of 5% as reimbursement of the payments that the Company made to settle a portion of the agreements.

These conditions raise substantial doubt about the Company's ability to continue as a going concern. These consolidated financial statements do not include any adjustments relating to the recovery of assets or the classification of liabilities that might be necessary should the Company be unable to continue as a going concern.

Management believes the Company's ability to continue its operations depends on its ability to raise capital. The Company entered into a licensing agreement providing it with the use of certain technology. The Company is currently developing pre-clinical and clinical studies of drug candidates. The licensing agreement described in Note 4

also enables the Company to sell the licensed technology as a research product or sublicense the technology to other third parties as alternative sources of revenue to its own product development efforts. The Company's future depends on the costs, timing, and outcome of regulatory reviews of its product candidates and the costs of commercialization activities, including product marketing, sales and distribution. During the first quarter of 2013, the Company has raised approximately $9.95 million in certain private placement transactions. The Company expects to continue to finance its cash needs through additional private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient

advocacy groups, foundations and government agencies. Although management believes that the Company has access to capital resources. there are no commitments for financing in place at this time, nor can management provide any assurance that such financing will be available on commercially acceptable terms, if at all.

NOTE 3.

- SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

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

Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiary in conformity with U.S. GAAP. All intercompany accounts and transactions have been eliminated in consolidation.

Restatement of Previously Issued Financial Statements for Additional Disclosures

The Company, while undergoing a review of its condensed consolidated financial statements for the three and six months periods ended June 30, 2013, commenced an evaluation of its accounting for a series of settlement agreements that the Company, along with certain of its related parties, entered into between April 2013 and June 2013. These agreements, which Company management originally deemed to be the primary obligation of a related party, are more fully described in Notes 2 and 12. On September 13, 2013, Company management, under the authority of the board of directors, determined that these agreements should have been disclosed in the footnotes to its consolidated financial statements for the year ended December 31, 2012. Accordingly, the Company has restated the consolidated financial statements to include these disclosures.

The Company also determined that its obligation to pay liquidated damages under a registration payment that it entered into in connection with a financing transaction completed on February 14, 2013, which required the Company to cause a registration statement to be declared effective by the Securities and Exchange Commission by May 15, 2013, should have also been disclosed. Accordingly, the Company is restating these consolidated financial statements to disclose that it allocated approximately $360.000 of the proceeds received in this financing transaction to a registration payment obligation that was deemed probable at the date that the financing transaction was completed.

Cash and Cash Equivalents

For purposes of the statement of cash flows, the Company considers cash instruments with maturities of less than three months when purchased to be cash equivalents. There are no cash equivalents as of the balance sheet date.

Property and Equipment

Property and equipment are stated at cost. Depreciation is provided for using the straight-line method over the estimated useful lives of the assets. At December 31, 2012 and 2011, property and equipment consisted of computers with an estimated useful life of three years and leasehold improvements with an estimated life of four years.

Employee Stock-Based Compensation

The Company accounts for stock-based compensation in accordance with ASC 718 Compensation — Stock Compensation (“ASC 718”). ASC 718 addresses all forms of share-based payment (“SBP”) awards including shares issued under employee stock purchase plans and stock incentive shares. Under ASC 718 awards result in a cost that is measured at fair value on the awards’ grant date, based on the estimated number of awards that are expected to vest and will result in a charge to operations.

Non-Employee Stock-Based Compensation

The Company accounts for equity instruments issued to non-employees in accordance with the provisions of ASC 505, Share Based Payments to Non-Employees, and ASC 718 which requires that such equity instruments are recorded at their fair value on the measurement date. The measurement of stock-based compensation is subject to periodic adjustment as the underlying equity instruments vest. Non-employee stock-based compensation charges are being amortized over their respective contractual vesting periods.

Income Taxes

The Company accounts for income taxes under ASC 740 Income Taxes ("ASC 740"). ASC 740 requires the recognition of deferred tax assets and liabilities for both the expected impact of differences between the financial statements and tax basis of assets and liabilities and for the expected future tax benefit to be derived from tax loss and tax credit carry forwards. ASC 740 additionally requires a valuation allowance to be established when it is more likely than not that all or a portion of deferred tax assets will not be realized.

ASC 740 also clarifies the accounting for uncertainty in income taxes recognized in an enterprise's financial statements and prescribes a recognition threshold and measurement process for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. For those benefits to be recognized, a tax position must be more-likely-than-not to be sustained upon examination by taxing authorities. ASC 740 also provides guidance on derecognition, classification, interest and penalties, accounting in interim periods, disclosure and transition. Based on the Company's evaluation, it has been concluded that there are no significant uncertain tax positions requiring recognition in the Company's unaudited financial statements. Since the Company was incorporated on March 11, 2011, all of its years of operations will be subject to examination. The Company believes that its income tax positions and deductions would be sustained on audit and does not anticipate any adjustments that would result in a material changes to its consolidated financial position.

The Company's policy for recording interest and penalties associated with audits is to record such expense as a component of income tax expense. There were no amounts accrued for penalties or interest as of or during the period from March 11, 2011 (inception) through December 31, 2012. Management is currently unaware of any issues under review that could result in significant payments, accruals or material deviations from its position.

Prior to conversion into a corporation on September 20, 2012, as a limited liability company, the Company was treated as a partnership for Federal and state income tax purposes. Accordingly, no provision has been made for Federal and state income taxes in the accompanying financial statements for any periods preceding September 20, 2012, since all items of income or loss are required to be reported on the income tax returns of the members, who are responsible for any taxes thereon. Profits and losses are allocated based upon capital in accordance with the permissible methods under Internal Revenue Code Section 706. Further, the Company incurred losses since inception through September 20, 2012, that would have resulted in the recognition of deferred tax assets that would have been fully reserved had the Company been subject to income taxes.

The Company is subject to the New York City Unincorporated Business Tax through September 19, 2012. Subsequent to Company's conversion to a corporation from a limited liability company on September 20, 2012, the Company will report and pay taxes based on its income or loss.

Use of Estimates

In preparing financial statements in conformity with accounting principles generally accepted in the United States of America, management is required to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting period. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions. These estimates and assumptions include valuing equity securities in share-based payments, estimating the useful lives of depreciable and amortizable assets and estimating the fair value of long-lived assets to assets whether impairment charges may apply.

Foreign Currency Translation and Remeasurement

Under ASC 830 Foreign Currency Matters, functional currency assets and liabilities are translated into the reporting currency, US Dollars, using period end rates of exchange and the related translation adjustments are recorded as a separate component of accumulated other comprehensive income. Functional statements of operations amounts expressed in functional currencies are translated using average exchange rates for the respective periods. Remeasurement adjustments and gains or losses resulting from foreign currency transactions are recorded as foreign exchange gains or losses in the consolidated statements of operations.

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Research and Development Costs:
Research and development costs are charged to operations as incurred and consist primarily of consulting services. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011 and for the period from March 11, 2011 (inception) through December 31, 2012, the Company incurred approximately $524,000, $353,000, and $877,000, respectively, relating to research and development costs that are included in professional fees in the accompanying consolidated statements of operations.
Patents
The Company capitalized external cost, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company expense cost associated with maintaining and defending patents subsequent to their issuance in the period incurred. The Company amortizes patent cost once issued on a straight-line basis over the estimate useful lives of the patents. The Company assess the potential impairment to all capitalized patent cost when events or changes in circumstances indicate that the carrying amount of our patent may not be recoverable. For the years ended December 31, 2012 and 2011 patents costs $18,093 and $0, respectively, are included in the accompanying consolidated balance sheets.
Basic and diluted Net Loss Per Share
Basic and diluted net loss per share has been computed by dividing net loss by the weighted average number of common shares outstanding during the period. All potentially dilutive common shares have been excluded since their inclusion would be anti-dilutive.
An aggregate of 267,768 and 1,602,390 common stock equivalents (incentive shares) were excluded from the computation of diluted net loss per common share for the year ended December 31, 2012 and for the period from March 11, 2011 (inception) through December 31, 2011, because they were contingent shares subject to recall.
Recently Issued Accounting Pronouncements
In February 2013, the FASB issued Accounting Standards Updated (“ASU”) 2013-04 “Obligations Resulting from Joint and Several Liability Arrangements for Which the Amount at the Reporting Date is Fixed”) (“ASU 2013-04”). The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount the entity has (a) contractually agreed to pay, and (b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company early adopted this guidance in the second quarter of 2013 (Note 12).
Except as noted above, management does not believe that any recently issued, but not yet effective accounting pronouncements, if adopted, would have a significant effect on the accompanying consolidated financial statements.
NOTE 4.

- ACCRUED EXPENSES

Accrued expenses consist of the following at December 31, 2012 and for the period from March 11, 2011 (inception) through December 31, 2011:

	December 31, 2012	**March 11, 2011 through December 31, 2011**
Compensation related costs	$1,022,716	$—
Consulting fees	679,800	169,721
Legal fees	563,380	—
Finders’ fee liability	100,000	—
Interest	90,650	—

	December 31, 2012	March 11, 2011 through December 31, 2011
Other	11,250	—
	$2,467,796	$169,721

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NOTE 5.

- LICENSE AGREEMENT

On February 16, 2012 the Company entered into an agreement pursuant to which a biotech company ('the Sublicensor') with license rights to certain drug technologies agreed to grant us a worldwide sublicense for the development, manufacture and commercialization of DARA, an ARB and ERA which we are initially using in connection with the treatment of FSGS and which we refer to as RE-021. The licensing agreement also enables the Company to sell the licensed technology as a research product or sublicense the technology to other third parties as potential sources of revenue. Under the license agreement, Sublicensor is obligated to transfer to the Company certain information, records, regulatory filings, materials and inventory controlled by Sublicensor and relating to or useful for developing RE-021. The Company must use commercially reasonable efforts to develop and commercialize RE-021in specified major market countries and other countries in which the Company believes it is commercially reasonable to develop and commercialize such products. The agreement shall continue until neither party has any obligations under the agreement to make payments to the other party.

In accordance with the agreement as amended most recently as of January 7, 2013, the Company is obligated to make two non-refundable payments totaling $2,450,000, the first payment of $1,150,000 due upon execution and the second payment of $1,300,000 due January 31, 2013, which includes a $150,000 fee payable to the sublicensee in exchange for extending due date of this payment from October 1, 2012 to January 31, 2013. If the Company makes the second payment after January 31, 2013 but before February 28, 2013 the payment due is $1,400,000, if before March 31, 2013 the payment due is $1,450,000. As of December 31, 2012, the Company has recognized $2,300,000 for the cost of the License Agreement which is presented in the accompanying consolidated balance sheet as an intangible asset that is being amortized on a straight-line basis over the term of the License Agreement which expires on September 30, 2023. As of December 31, 2012, the Company made one payment of $1,150,000. The Company has recorded a $1,300,000 liability in the accompanying consolidated balance sheet at December 31, 2012 for the remaining payment of $1,150,000 plus $150,000 of extension fees. In addition, as more fully described below, the Company issued 620,000 common shares to Ligand valued at $1,550,000 as a result of the merger transaction. For the year ended December 31, 2012, the Company recognized amortization expense of the license related to this agreement totaling $121,383.

In addition, the Company is obligated to make series of milestone payments upon the achievement of each development milestone events set forth in the sublicense agreement which could amount to an aggregate of up to $106.9 million. Milestone payments as they become due will be recognized as license expense, pro-rata over the period through September 2023.

Per the sublicense agreement, starting from the first commercial sale of any licensed product (as defined in the agreement), the Company is obligated to pay the Sublicensor royalty payments equal to 15% of annual worldwide net sales of licensed product up to $300,000. For worldwide net sales of licensed product exceeding $300,000, a royalty percentage of 17% is applied. Royalties are payable on a quarterly basis, and are payable on a product-by-product and country-by country basis on the net sales of licensed products. Royalties terms will be in effect until the later of (i) ten years after the first commercial sale of any licensed product in such country or (ii) the expiration of any patent rights licensed under the license agreement (iii) the expiration of all periods of market exclusivity. Currently, the last to expire issued patent covered by such arrangement expires in September 2023; however, the Company expects such date may be extended by patent-term extensions. The sublicense agreement contains other customary clauses and terms as are common in similar agreements in the industry.

In the event the Company's Exit Transaction defined in the agreement as (i) sale of all or substantially all of the Company's assets or business or (ii) a merger, reorganization or consolidation involving the Company in which the stockholders or members of the Company immediately prior to such transaction cease to own collectively a majority of the voting equity securities or membership interests of a successor entity or (iii) a registered public offering of

Company’s common stock under the Securities Act of 1933 or (iv) a reverse merger of Company into an existing public company), the Company is obligated to pay the Sublicensor $1,500,000 no later than fifteen business days prior to the closing of the Exit Transaction. The Company has an option to issue capital stock in lieu of a cash payment to the Sublicensor. Should the Company choose to issue capital stocks, the number of shares of capital stock issue shall be equal to $1,500,000 divided by the per share price of the capital stock to be agreed upon between the Company and the Sublicensor on the date such election is made.

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NOTE 6.

- NOTES PAYABLE

Note Payable—related party
On February 1, 2012, the Company entered into a secured promissory note with a related party in the amount of $900,000, with an interest rate of 12% per annum, compounded monthly. The note plus accrued unpaid interest shall become due (i) on or prior to December 31, 2012 or (ii) upon consummation of a Sale of the Company to acquire (a) a majority of the outstanding equity securities, or (b) all or substantially all of the Company's assets on a consolidated basis.
In addition, the Company has the right to repay a portion of the outstanding obligation without penalty or premium. The repayment amount shall be applied in the following order: (i) any expenses to be reimbursed to the related party, (ii) all unpaid interest through the date of repayment and (iii) against the principal amount. On March 5, 2012, an aggregate payment of $25,000 was made by the Company, of which $9,764 was applied to accrued interest and the remaining balance of $15,236 was applied to the principal balance. The remaining principal balance of this note amounts to $884,764 as of December 31, 2012. The remaining principal balance of the note was repaid subsequent to year end.
On December 28, 2012, the secured promissory note was extended to June 30, 2013.
Note Payable—employee
On September 30, 2012, the Company received an advance of $30,000 from a related party in the form of a promissory note, with an interest rate of 15% per annum, compounded monthly. The note expired on the earlier of (i) December 31, 2012 or (ii) upon a significant change in the Company's ownership (as defined in the promissory note). On December 3, 2012, the Company repaid $30,000 plus any unpaid interest.
The accrued interest payable related to the two notes payable at December 31, 2012 and 2011 was aggregated to $90,650 and $0, respectively.
Total interest expense recognized for the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011 and for the period from March 11, 2011 (inception) through December 31, 2012 were aggregated to $105,917, $0 and $105,917, respectively.
NOTE 7.

- RELATED PARTY TRANSACTIONS

In October and November 2011, the Company was advanced $7,500, from a company related through common ownership. The advance is due on demand.
In November 2011, the Company was advanced $30,000 from a company related through common ownership. The advances were repaid in February 2012.
On December 8, 2011, the Company received advances of funds aggregating $8,500 from entities related through common ownership. The advances are due on demand. Balance remaining at December 31, 2012 was $5,700.
In August 2012, the Company paid a security deposit on behalf of an affiliate of $137,547 in connection with a building lease entered into by such affiliate. The Company assumed the lease from its affiliate in April 2013, whereby the security deposit was assigned to the Company.
During the year 2012, the Company paid an aggregate amount of $563,380 in legal fees on behalf of the same affiliate. The affiliate is currently in the process of dissolving and the Company does not expect to collect the amount outstanding. As a result, the Company has written-off $563,380 to bad debt expense in 2012. Such charge is included in selling general and administrative expense in the statement of operations. Subsequent to December 31, 2012, the Company became a party to certain settlement agreements, which management believes are the primary obligation of this affiliate (Note 12).

NOTE 8.

- STOCKHOLDERS' DEFICIT

Post-Merger Capitalization with Desert Gateway
Common Stock
The Company is currently authorized to issue up to 100,000,000 shares of $0.0001 par value common stock. All issued shares of common stock are entitled to vote on a 1 share/1 vote basis.
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Preferred Stock
The Company is currently authorized to issue up to 20,000,000 shares of $0.001 preferred stock, of which 1,000 shares are designated Class “A” Preferred shares, $0.001 par value. Class A Preferred Shares are not entitled to interest, have certain liquidation preferences, special voting rights-and other provisions. No Preferred Shares have been issued to date.
Issuances
Common Stock
On March 30, 2011, the Company issued to its founder 1,608,300 shares of Common Stock for a $25,000 capital contribution.
On March 31, 2011, the Company issued to a member 50,000 shares of Common Stock for a $100 capital contribution.
Private Placement Offering—March 2011
On March 31, 2011, the Company offered for sale, pursuant to a Private Placement Memorandum (“PPM”), up to 500,000 of the Company’s Common Stock at $4 per share, for an aggregate offering price of $2,000,000. The Common Stock was entitled to one (1) vote per each unit outstanding. The termination date of this offer was originally May 3, 2011. On June 15, 2011, the Company extended the termination date of the PPM to August 31, 2011.
In April, May and June 2011, the Company sold shares of Common Stock in a private placement for $4 per share, yielding aggregate proceeds of $725,000. In addition, the Company incurred aggregate fees of $66,061 in connection with the private placement. These common shares were subsequently exchanged for Series A Preferred shares (subsequently recapitalized into 253,750 shares of common stock).
Incentive Stock Awards
Since Inception, the Company entered into various incentive unit agreements for issuances of Incentive Common Shares with certain individuals for future services (see note 9).
Preferred Stock
On June 30, 2011, the Company amended its PPM to sell a new series of units of membership interest known as the “Series A Preferred Stock,” instead of common stock. The Series A Preferred Shares have a liquidation priority over the Common Shares with a preference equal to two (2) times the amount originally invested in such shares (including any prior cash distributions of any operating profits) before any amounts are paid with respect to any Common Stock. In conjunction with the amended PPM, the Company amended the subscription agreements of the prior Common Stockholders and changed the Stock ownership to the newly issued Series A Preferred Stocks.
In July, October and December 2011, the Company sold shares of Series A Preferred Stock (subsequently recapitalized into 36,750 shares of common stock) related to the amended private placement for approximately $2.86 per share, yielding aggregate proceeds of $105,000 of which 10,500 shares sold and $30,000 proceeds were from a related party through common ownership. In addition, the Company incurred aggregate fees of $1,367 in connection with the private placement.
On January 25, 2012, the Company, in connection with a January 2012 private placement offered for sale up to 875,000 shares of the Company’s Series A Preferred Shares at approximately $5.71 per share with similar terms and conditions as the amended PPM.
From January 1, 2012 through May 14, 2012, the Company sold shares of Series A Preferred Stock (subsequently recapitalized into 326,963 shares of common stock) related to the January 2012 private placement at approximately $5.71 per Share, yielding aggregate proceeds of $1,868,354 of which 128,163 shares sold and $732,353 proceeds were from a related party through common ownership. In addition, the Company incurred aggregate fees of $61,677 in connection with the private placement.
On May 18, 2012, the Company, in connection with the May 2012 private placement, offered for sale up to 875,000 shares of the Company’s Series A Preferred Stock at approximately $11.43 per share with similar terms and conditions as the amended PPM.

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On September 20, 2012, the Company amended its May 2012 private placement selling price of the Preferred Shares from approximately $11.43 per share to approximately $3.57 per share as a result of a resolution of the Company's board. This resolution was determined as a result of market conditions.

From May 31, 2012 through September 25, 2012, the Company sold shares of the Series A Preferred Stock (subsequently recapitalized into 271,824 shares of common stock) related to May 2012 private placement at approximately $3.57 per share, yielding aggregate proceeds of $970,800 of which 185,024 shares sold and $660,800 proceeds were from a related party through common ownership. In addition, the Company incurred aggregate fees of $12,275 in connection with the private placement.

From October 1, 2012 through December 11, 2012, the Company sold shares of the Series A Preferred Stock (subsequently recapitalized into 198,940 shares of common stock) related to May 2012 private placement at approximately $3.57 per Unit, yielding aggregate proceeds of $710,501.

Capital Contributions of Common Shares by Founder

In April 2012, the Company's founding stockholder personally transferred 300,000 shares of his common stock to a third-party consultant for advisory services provided to the company. In September 2012, the Company's founder personally transferred 250,000 shares of his common stock to the former Chief Executive Officer and current Chairman of the Board of Directors. The shares in both of these transactions, which have an aggregate fair value of $4,400,000, are fully vested and non-forfeitable.

In November 2012 and December 2012, the Company's founding shareholder personally transferred 275,000 shares of his common stock to several employees. The shares, which had an aggregate fair value of $1,375,000, are fully vested and non-forfeitable.

Receivables from Shareholders

In November of 2011, the Company advanced $10,000 to a related party, with an interest rate of 0.001% and a five year term. The advance is classified as a note receivable from related party on the balance sheet at December 31, 2011 and is due on November 3, 2016, the note is classified as a reduction of stockholders' equity in the accompanying consolidated balance sheet.

On February 3, 2012, the Company entered into a note receivable with a related party in the amount of $200,000. The note receivable is unsecured, bearing an interest rate of 12% per annum and due to mature on February 3, 2013. The note is classified as a reduction of stockholders' equity in the accompanying consolidated balance sheet.

Advances to shareholders consist of payments made by the Company for entities commonly controlled by the shareholders for operating expenses aggregating $172,900.

NOTE 9.

- INCENTIVE SHARES

On March 31, 2011, the Company granted 1,849,300 incentive shares to several executive and non-executive employees, and certain consultants, with an aggregate fair value of $7,397,200 or $4 per share. The incentive shares vested on the final day of each calendar quarter over three years, commencing on June 30, 2011. On September 11, 2012, the Company accelerated the vesting of 938,175 shares issued to its founder and Chief Executive Officer, which resulted in a charge of $3,216,600 included in compensation and related costs in the accompanying statement of operations.

In August and November 2011, the Company granted an aggregate of 290,000 incentive shares to two consultants, with an aggregate fair value of $1,160,000 or $4 per share, for consulting services. The incentive shares vested on the final day of each calendar quarter over three years, commencing on June 30, 2011 and December 31, 2011.

In January 2012, the Company granted 826,600 incentive shares to the Chief Executive Officer, an employee and a consultant, with an aggregate fair value of $9,919,200 or $12 per share. The incentive shares vested on the final day of each calendar quarter over three years, commencing on March 31, 2012. On September 11, 2012, the Company immediately vested the Chief Executive Officer's unvested incentive shares totaling 28.185 for continuing services. On

December 11, 2012, the Chief Executive Officer's remaining unvested incentive shares totaling 573,015 were vested immediately due to the merger, which resulted in an aggregate charge of $7,214,400 included in compensation and related costs in the accompanying statement of operations.

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On March 7, 2012, the Company granted 83,333 incentive shares to a third party consultant, with an aggregate fair value of $2,000,000 or approximately $24 per share, for consulting services. The incentive shares vested (i) 50% immediately and (ii) on the final day of each calendar quarter over two years, commencing on March 31, 2012.
On July 7, 2012, the Company granted 43,750 incentive shares to an employee, with an aggregate fair value of $375,000 or approximately $8.6 per share. The incentive shares vested on the final day of each calendar quarter over three years, commencing on September 30, 2012.
For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and for the period from March 11, 2011 (inception) through December 31, 2012, the Company recognized $22,410,222, $1,979,299, and $24,389,521 as compensation expense related to incentive shares granted in the consolidated statements of operations, respectively. Share compensation for non-employee awards subject to vesting is being accrued at current fair value as of December 31, 2012, there was approximately $844,973 of unrecognized compensation cost related to incentive shares issued. This amount is expected to be recognized over a weighted average of 1.69 years.

	Employee – number of shares	**Non Employee – number of shares**	**Total number of shares**	**Weighted Average Fair Value**
Unvested March 11, 2011 (“inception”)				$—
Granted	1,758,300	381,000	2,139,300	4.00
Vested	(431,240)	(59,835)	(491,075)	4.00
Forfeited	(45,835)	—	(45,835)	—
Unvested December 31, 2011	1,281,225	321,165	1,602,390	$4.00
Granted	866,180	87,503	953,683	12.89
Vested	(2,048,280)	(193,672)	(2,241,952)	7.34
Forfeited	(46,353)	—	(46,353)	—
Unvested December 31, 2012	52,772	214,996	267,768	$3.20

All of the Company’s share base payments were originally issued as Retrophin LLC Class B incentive units that represent a profits interest up through the date of the Retrophin’s conversation to a C Corporation, which was structured as a tax free exchange transaction.
Shares granted as incentive shares were originally subject to certain conditions at the time of grant. Such conditions specified that the occurrence of a Termination Event, as defined in the amended operating agreement the Company shall have the right, but not the obligation, to repurchase, all, of the vested incentive shares owned by such incentive shareholder, at a purchase price based on the fair market value of the incentive shares determined in good faith by the Board of Directors. The aforementioned repurchase option was rescinded upon the Company’s conversion to a corporation.
NOTE 10.

- COMMITMENTS AND CONTINGENCIES

Sublease
During March 2011, the Company began subleasing offices on a month-to-month basis for $7,000 per month. On June 31, 2011, the Company entered into a sublease agreement with a company affiliated by common ownership, where the Company will pay $7,000 a month or 75% of the space used, pro-rated, according to the aggregate cost of the shared offices with the affiliated entities of the related party leasing company, whichever is greater. According to

the agreement, the Company is responsible for incidental costs and for rent or lease of office furniture and equipment. The sublease is on a six month rolling basis and termination of the agreement can be made by a mutual agreement of both parties or by the related party leasing company. The month-to-month lease was terminated in September 2012.

In October 2012, the Company entered into a sublease with a company ("Sublessor") affiliated by common ownership that expires on November 29, 2016. The sublease agreement requires the Company to pay 50% of the rent and related escalations and for the Company to pay for 50% of the utilities incurred by the Sublessor.

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Rent expense for the year ended December 31, 2012, for the periods from March 11 2011 (inception) through December 31, 2011 and from March 11, 2011 (inception) through December 31, 2012 were $95,469, $63.000 and $158.469, respectively, which is recorded as rent expense in the consolidated statement of operations.
As of December 31, 2012 minimum future rental commitments under non-cancelable operating leases follow:

Year Ending December 31,	
2013	$138,200
2014	140,161
2015	140,161
2016	128,481
Total	$547,003

On April 11, 2013, the lease was assigned to the Company by the Sublessor inclusive of the security deposit held.
Consulting Agreements
On August 15, 2011, the Company entered into an agreement with a consultant to serve as a senior advisor of strategy. The agreement's initial term is for one year and automatically renews on an annual basis. Pursuant to this agreement the compensation to the consultant is comprised of (a) a fee of $37,500 per calendar quarter, payable commencing September 30, 2011, (b) 25,000 shares of the Company Common Stock with an estimated fair value of $100,000, which vest over twelve (12) quarters so long as the agreement remains in effect, and (c) 25,000 additional common stock, (i) upon the Company's completion of its initial financing at a pre-financing value of $20 million, and (ii) which vest in accordance with certain schedules of milestones as described in the consulting agreement. At December 31, 2012, the financing and milestones have not yet occurred or been achieved. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and December 31, 2012, the Company recognized professional fees related to this agreement in the amounts of $150,000, $75,000, and $225,000, respectively, of which amounts comprised of fee payable of $155,000 and $75,000 at December 31, 2012 and 2011, respectively.
On November 1, 2011, the Company granted to the consultant an additional 120,000 shares of common stock with an estimate fair value of $480,000, which vest in over twelve (12) calendar quarters commencing December 31, 2011. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and for the period from March 11, 2011 (inception) through December 31, 2012, the Company recognized professional fees related to this share based compensation of $210,000, $40,000, and $250,000.
On August 25, 2011, the Company entered into an agreement with a consultant to serve as chief scientific officer of the Company.
The agreement's initial term is for one year and automatically renews on an annual basis. Pursuant to this agreement the compensation to the consultant is comprised of (a) a fee of $50,000 per calendar quarter, (b) 75,000 incentive shares with an estimated fair value of $300,000, which vest over twelve (12) quarters so long as the agreement remains in effect, and (c) receive 70,000 additional incentive shares, (i) upon the Company's completion of its initial financing at a prefinancing value of $20 million, and (ii) which vest in accordance with certain schedules of milestones as described in the consulting agreement. At December 31, 2012, the financing and milestones have not yet occurred or been achieved. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and for the period from March 11, 2011 (inception) through December 31, 2012, the Company recognized professional expense related to this agreement in amounts of $200,000, $100,000, and 300,000, respectively, of which amounts comprise of fee payable of $200,000 and $100,000 at December 31, 2012 and December 31, 2011, respectively.
On November 1, 2011, the Company granted to the consultant an additional 70,000 incentive shares with an estimated fair value of $280,000, which vest in over twelve (12) calendar quarters commencing December 31, 2011. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and for the

period from March 11, 2011 (inception) through December 31, 2012, the Company recognized professional expense related to this share based compensation of $122,500, $23,333, and $145,833.

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Sponsored Research Agreement
On July 1, 2012, the Company entered into a Sponsored Research Agreement with an organization that expires on July 1, 2013, unless extended by written agreement between the parties. The Company has agreed to pay a sponsor fee of $203,169 to the organization to perform the research program stated in the Sponsored Research Agreement. The sponsor fee payments are as follows: $101,855 within 30 days of the execution of the agreement and the remaining $101,314 will be due on January 1, 2013. As of December 31, 2012, the Company included the first payment of $101,855 in accounts payable and accrued expenses, as no payment have been made by the Company.
Sponsor fee totaling $203,169 will be recognized as professional expense, pro-rata over the one year term of the Sponsored Research Agreement. Total professional expense recorded related to the Sponsored Research Agreement totaled $101,855 for the year ended December 31, 2012.
Employment agreement
Effective March 1, 2011, the Company entered into a three-year employment agreement with Martin Shkreli, who serves as the Company's Chief Executive Officer. The Agreement provides for (a) a base salary of $250,000 per year, (b) annual cash bonus award at the discretion of the Board equal to one month salary, (c) three weeks' vacation paid per calendar year, (d) accelerated vesting of options in the event of (i) a merger or consolidation, (ii) a sale of all or substantially all of the assets or (iii) any other change in control of the Company, and (e) all group insurance plans and other benefit plans and programs made available to the Company's management employees.
NOTE 11.

- INCOME TAXES

From the Company's inception in March 11, 2011 to September 20, 2012, the Company was not subject to federal and state income taxes since it was operating as a Limited Liability Company (LLC). On September 20, 2012, the company converted from an LLC to a C corporation and, as a result, became subject to corporate federal and state income taxes. This conversion is considered a recapitalization of the equity structure of the company and was treated as a nontaxable transaction. As a result of the conversion to a taxable entity, the Company recorded a deferred tax liability on the balance sheet and in income tax expense as of the date of the change in tax status in the amount of $1,079,000 related to the technology license. The company files its taxes on a cash basis method.
For the period ended December 31, 2012, the Company incurred net operating losses and, accordingly, no provision for income taxes has been recorded. In addition, no benefit for income taxes has been recorded due to the uncertainty of the realization of any tax assets including NOL carryovers. At December 31, 2012, the Company had approximately $5.9 million dollars of federal and state and local net operating losses. The net operating loss carry forwards, if not utilized, will begin to expire in 2032 for federal purposes.
The components of the provision (benefit) for income taxes, in the consolidated statement of operations are as follows (in thousands):

	2012
Current	
Federal	$—
State	—
	—
Deferred	
Federal	(1,173)
State	(733)
	(1,906)
Total	$(1,906)
Change in valuation allowance	1,906

	2012
Income tax (benefit)	—
Total	$—

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A reconciliation of the statutory federal income tax expense (benefit) to the effective tax is as follows (in thousands):

	2012	
Statutory rate—federal	(35.00	%)
State taxes, net of federal benefit	(1.81	%)
Partnership Losses preceding the conversion to a C Corp	19.39	%
Stock Based Compensation related to profits interest	9.52	%
Meals & Entertainment	0.01	%
Deferred tax adjustment upon conversion to taxable status	1.61	%
Change in valuation allowance	6.28	%
Income tax provision (benefit)	0.00	%

The tax effects of “temporary differences” give rise to deferred tax assets and liabilities as of December 31, 2012. (in thousands):

	2012	Asset	Liability
Net operating loss and capital loss carry forward	$2,748	$2,748	
Technology license	$(466)		(466)
Organizational costs	$9		9
Accrual to Cash	$(385)		(385)
Valuation allowance	$(1,906)		
Total	$—	$2,748	$(842)

NOTE 12.

- SUBSEQUENT EVENTS

In January 2013, Desert Gateway Inc. sold an aggregate of 272,221 shares of common stock in certain private placement transactions, for an aggregate purchase price of $816,664 in cash. The issuance of such shares of common stock was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

On February 14, 2013, Desert Gateway Inc. closed a private placement of 3,045,929 shares of Desert Gateway Inc. common stock, at a purchase price of $3.00 per share, or $9,137,787 in the aggregate, and Warrants to purchase up to an aggregate of 1,522,969 shares of common stock with an exercise price of $3.60 per such share underlying any Warrant. The issuance of the shares of common stock in such private placement was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

The Company concurrently entered into a registration payment arrangement requiring it to file a Form S-1 with the Securities and Exchange Commission within 30 days of the closing date of this transaction and cause it to be declared effective by no later than 90 days of the closing date of this transaction. The registration payment arrangement provided for the Company to pay liquidated damages in the amount of 2% of the proceeds received in this transaction per month for each month that the Company is not in compliance with this requirement, not to exceed 10% in the aggregate. The Company determined that it was probable that it would not be in a position to comply with these requirements and therefore allocated approximately $360,000 of the proceeds received in this transaction to a registration payment obligation.

Effective May 13, 2013, the Company entered into an employment agreement with Horacio Plotkin, M.D. (the “Plotkin Employment Agreement”) pursuant to which Dr. Plotkin was appointed as Chief Medical Officer of the Company.

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In accordance with the terms of the Plotkin Employment Agreement, Dr. Plotkin's initial base salary is $350,000 and he is eligible to receive a discretionary annual bonus of up to 50% of his then applicable base salary. Additionally. Dr. Plotkin received $20,000 in connection with signing the Plotkin Employment Agreement. Dr. Plotkin will also be awarded options to purchase 120,000 shares of restricted common stock of the Company at an exercise price of $8.70 per share, a pro rata portion of which will vest quarterly during the 3 years following the effective date.

Effective May 20, 2013, the Company entered into an employment agreement with Marc L. Panoff (the "Panoff Employment Agreement") pursuant to which Mr. Panoff was appointed as Chief Financial Officer and Chief Accounting Officer of the Company.

In accordance with the terms of the Panoff Employment Agreement, Mr. Panoffs initial base salary is $230,000 and he is eligible to receive a discretionary annual bonus of up to 50% of his then applicable base salary. Mr. Panoff will also be granted 120.000 units of restricted common stock of the Company, a pro rata portion of which will vest quarterly during the 3 years following the effective date.

In the second quarter of 2013, the Company, its Chief Executive Officer and a related party became party to a series of agreements to settle up to $2,284,511 of liabilities, which Company management believes are the primary obligation of the related party. The Company and the related party have entered into indemnification agreements whereby the related party has agreed to defend and hold the Company harmless against all such obligations and amounts, whether paid or unpaid, arising from these agreements. Notwithstanding the indemnification, the Company recorded a $2,284,511 charge to operations during the quarter ended June 30, 2013 that was offset by a corresponding liability of $1,691,400 for the difference between (a) the aggregate amount of all such settlements, and (b) $593,111 of cash and non-cash consideration that the Company paid to immediately settle a portion of the agreement on behalf of the related party. Of the total outstanding $1,691,400 settlement liability, $300,000 is past due, $713,900 was due in July 2013, and $677,500 was due in August 2013. The counter parties to these agreements reserve the right to demand payment at any time. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock in the Company to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the second quarter ended June 30, 2013. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

Concurrent with the execution of such settlement agreements, the Company received promissory notes from the related party whereby the related party agreed to pay the Company the principal amount of $593,111 plus interest at an annualized rate of 5% as reimbursement of the payments that the Company made to settle a portion of the agreements.

The Company applied the accounting guidance provided in ASU 2013-04. The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount that the entity (a) has contractually agreed to pay, and (b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company has recorded the full amount of the settlements as a charge to its operations due to uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary. Any amounts that the Company may recover under the note due from the related party or under the terms of the indemnification agreement, if in fact any amounts are recovered at all, would be characterized as a capital contribution at the date such payments are received.

In accordance with ASC 855-10, Company management reviewed all material events through the date of this report and there are no material subsequent events to report, other than those listed in the Note.

TABLE OF CONTENTS

4,705,882 Shares
Retrophin, Inc.
Common Stock

P R O S P E C T U S

Sole Book-Running Manager
Jefferies
Co-Managers
Roth Capital Partners
Ladenburg Thalmann & Co. Inc.
Summer Street Research Partners
January 10, 2014